

2019



ANNUAL REPORT | 2019

Care4BrittleBones Foundation



OI | IN NUMBERS

Some children break one of their bones

500.000 PEOPLE **10** TIMES A YEAR

Worldwide have OI

Germany: 5,000; UK, France and Italy: 4,000 each;
Netherlands: 1,000; Belgium: 700; Switzerland: 500

People most affected with OI break over

200 TIMES IN A LIFETIME

THE SHATTERING TRUTH

8.500 BABIES
0 MEDICATIONS

Worldwide are born each year with OI

Have been developed specially for OI

OI is one of

7.000 RARE DISEASES

Together rare diseases are the 4th largest
disease classification in the world.

320 VOLUNTEERS

522.800 EUR

2.2 %

Overhead

Spent and/or allocated to our goals (research,
information sharing, collaboration/knowledge
exchange for OI, including conference)

348.000 EUR

REACHING OUT IN 2019

Of funds raised in 2019 for much needed research for OI

12 FUNDRAISING EVENTS

Organised in 2019 by Care4BrittleBones and its network

3.7 %

Fundraising Cost



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2019 | DELIVERING ON AMBITIONS

We started the year with strong ambitions and with the confidence of knowing that in the recent past we had established firm foundations for the Charity (for example, joining Goede Doelen Nederland and attaining CBF certification) and achieved a number of key milestones (for example, surpassing €1 million raised to fund vital research). This provided us with a strong platform as we worked together to continue to improve quality of life for people with OI.

As you will read in the pages that follow, 2019 was a year with many highlights in terms of funds raised, in terms of the OI research agenda, and in terms of our role in the flagship “Key for OI” project. Perhaps the most prominent highlight was the international “QualityofLife4OI” conference which we organised in Amsterdam in November, bringing together hundreds of members of the global OI community including clinicians, researchers, allied health professionals, industry representatives and people with OI and other rare bone diseases. Special thanks to our colleagues in OIFE, OIF, ERN-BOND and BBDC who partnered with us to make this happen.

So we ended the year on something of a high note, knowing that we had delivered on our key ambitions. This provides us with real momentum moving into 2020, a year which we already see bringing its own significant challenges and unique uncertainties. Nevertheless, we look back on 2019 with pride in what we continue to achieve with our partners throughout the OI world, and with real gratitude for the encouragement, support and trust placed in us. We will continue to work with all our dedication and focus to make a difference to the quality of life for people with OI.

On behalf of the whole foundation Care4BrittleBones, thank you!

Graham Marshall
Chairman



Dagmar Mekking
CEO/Directeur-Bestuurder





WHAT IS OSTEOGENESIS IMPERFECTA (OI)?

Brittle Bone Disease, also called OI, is a genetic bone condition that affects the connective tissue of the body. It is caused by insufficient production of collagen that normally ensures strong, healthy bones.

*“We have a single goal:
to enable scientific
research that improves
the quality of life
of people with OI...
and ultimately find
the cure!”*

The main characteristic of OI is the fragility of the bones. People with OI can experience anything from “a few more fractures than normal” to more than 200 fractures in a lifetime. A small incident can have big consequences for people with OI. Other symptoms include deformities, chronic pain, scoliosis, short stature, hearing and dental problems as well as severe breathing and heart issues.

OI is a purely physical disorder but has broader consequences. People with OI have the same needs and hopes for their lives as everyone else but they need to realise their dreams with a very fragile body. Often OI-related physical challenges also lead to significant psychosocial challenges, like exclusion and the fear of experiencing yet another fracture. This can lead to people with OI feeling left out and alone.

There are five main types of OI. People with the disorder are affected in very different ways – those with a less severe form of OI are able to work and participate fully in study and social activities. Many people with OI, however, have to deal with frequent pain and mobility issues, which heavily influence how they live their lives. Some people with OI can have a significantly reduced life expectancy.

Children are the most heavily affected group. Some children will have a fracture every few weeks, undergo 20 surgeries or more at a young age and spend half their childhood wearing a cast. This has a big impact on the child as well as the family around him/her.

Generally, the most important symptoms of OI we are looking to improve are:

| Fractures | Pain | Anxiety | Isolation





| STATUS OF RESEARCH FOR OI TODAY

OI has been known for more than 100 years; however, there is no treatment that fundamentally addresses the disease. The key approaches used today are 'hand-me-down medications' from other related diseases, e.g. Osteoporosis. These medications treat some of the symptoms, for example by making the bones harder (using Bisphosphonates). Whilst these medications clearly have benefits, they also come with potential adverse side effects. Moreover their long-term effects are unclear. Most striking of all is that they don't address the root cause. Simply put: this is not good enough.

Research for OI patients is not providing the right solutions today because it is a **rare disease** affecting relatively few people. There are about 500.000 people worldwide with this disorder (1 person out of 15.000 has OI). Like many other rare diseases, hardly any funding for research is available to change the status quo.

It is time to address what is holding back progress. We are keen to put urgently needed support in place to address the challenges of OI in a strategic way. The key reasons for pushing forward now are:

1. **Promising new technologies** have become available, such as gene therapy or stem cell therapy, new medical treatment options, innovative materials and techniques for surgeries.
2. Modern **communication technology** is enabling collaboration and knowledge sharing between the world's top experts for rare diseases. Distributed around the world they previously worked mostly in isolation.
3. **Big data** for small diseases is coming within reach. Genetic testing has become widely accessible and is cheaper than ever before. Over time this will allow us to look at 'scientific mysteries' like the discrepancy between genotype and phenotype. The largest research ever done in OI addressed less than 3.000 people. Significantly larger cohorts will soon be possible. This will enable new insights into patterns and stronger research as long as researchers are willing to work together and share data and/or patient organisations work together to collect data themselves.
4. There are significant questions to be addressed regarding bone metabolism, which still has unknown territory. A new trend in the last 2-3 years is to **look across related rare bone diseases** to understand the underlying biochemical processes. This has led to the formation of the European Reference Network for Rare Bone Diseases and the US-Canadian Brittle Bone Diseases Consortium. OI within both of these groups is standing out as the top priority research area amongst the nearly 450 rare bone diseases that exist.

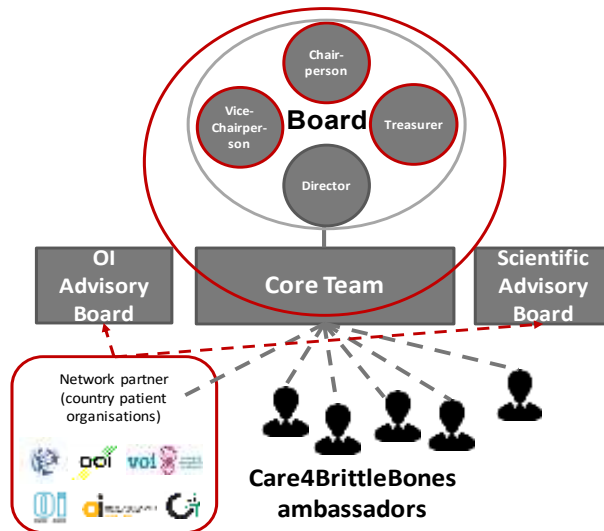
These conditions create new opportunities for OI research. Care4BrittleBones has identified various short, mid and long-term opportunities that have the potential to ameliorate quality of life for people with Osteogenesis Imperfecta and eventually to lead to a breakthrough therapy for OI.



CARE4BRITTLEBONES | ORGANISATION

Care4BrittleBones consists of five vital teams:

1. The Board
2. The Core Team
3. Two Advisory Boards
4. Our Ambassadors
5. Our Network Partners



1. | THE BOARD

Care4BrittleBones is formally led by its Board of four team members:



Graham Marshall
Chairman/Secretary



Celine Disch
Communications



Dirk-Jan Stegink
Treasurer



Dagmar Mekking
Director

The Board is ultimately accountable for all aspects of the foundation, with a strong focus on strategy, finance, risk and governance.

Governance of the foundation by the Board is organised as follows:

The Board meets on an ad hoc basis and approves the annual budget, operational plan, strategy document and (re)appointment of the director and monitors the performance against the plans. We also meet on a monthly basis together with the Core Team to discuss all matters both strategic and operational. The chairman and director meet weekly to consult on key topics. The Board will formally review the performance of the director annually.



2. | THE CORE TEAM

The activities of the foundation are carried out by the Core Team, which acts as an extension of the Board. They define the strategy and operational plans, set and manage standards for fundraising and research, drive communications and PR, manage the financials and compliance, manage projects, develop relationships with funding institutions, support volunteers and manage the annual research proposal rounds.

The Core Team consists of highly skilled and motivated professionals from multiple industry sectors with significant experience working internationally and cross-culturally. They meet on a monthly basis to discuss all matters concerning the foundation. The Core Team has various subcommittees:

Finance	Dirk-Jan Stegink , Iwona Juzwa-Parfus,
Fundraising	Dagmar Mekking , Lieke Mekking, Graham Marshall
Communications	Celine Disch , Ariel Slaughter, Chaya Mistry, Annet Snoek
IT / Data Privacy	Peter Kemper, Henk Latour , Kees Mezouar
Research	Graham Marshall , Dagmar Mekking, Lieke Mekking, Celine Disch
Collaboration	Dagmar Mekking , Graham Marshall





3. | TWO ADVISORY BOARDS

Advisory Board members are carefully chosen based on their expertise and reputation for collaboration. They are acting in the best interest of people with OI worldwide, irrespective of their own discipline or national background.

The Scientific Advisory Board includes expertise in genetics, endocrinology, bone metabolism, paediatrics and rehabilitation, for example. All scientific advisory board members have an outstanding professional reputation and a proven track record of working closely with the OI-community over many years.

All members of the OI Advisory Board have OI themselves and have good knowledge of the disorder and a broad network.

The Advisory Boards advise on which research projects we should prioritise and steer on research strategy and communication. In 2019 we did not hold a proposal round due to the conference Quality of Life 4 OI (see dedicated chapter in this Annual report), however almost every single person was actively involved in one or more research project taking place this year and participated actively in the conference QualityofLife4OI.

THE SCIENTIFIC ADVISORY BOARD



Fleur van Dijk



Oliver Semler



Lena Lande Wekre



Lars Folkestad



Marianne Rohrbach



Antonella Forlino



Geert Mortier

THE OI ADVISORY BOARD



Margriet Crezee
Netherlands



Ute Wallentin
Germany



Eero Nevalainen
Finland



Rebecca Tvedt
ERN-BOND
EPAG



Bruno van Dijk
Belgium



Therese Stutz-Steiger
Switzerland



Leonardo Panzeri
Italy



Benedicte Alliot
France



4. | OUR AMBASSADORS

Our ambassadors are dedicated to networking and fundraising for OI research. Most of them either have OI themselves or have a relative with OI. We encourage anyone around the world who would like to support Care4BrittleBones by raising awareness and fundraising to get in touch (e-mail to dagmar.mekking@care4brittlebones.org).



Andrea Kiel



Annemarie
Visser & Kris
Koenis



Danielle de
Bakker



Diana Lips



Erik Meinen



Harry
Vondenhoff



Leonie Rouweler



Irma van der
Hoek



Jeanette
Chedda



Jessy van de Pol



Karin Kragt-
Vermolen



Laura Marx



Marijke van
Liempt



Marina Gil



Melvin
Elderhorst



Michaël Roele



Mira Thompson



Rebecca Meijer



Rene van der
Rijst



Rick Brink



Roos Hoffmann



Rob & Rebecca
van Berkel



Welmoed
Santema



Zuhal Soyan

In addition, many children enthusiastically and effectively help us! They intuitively understand how difficult it is to be excluded from normal play and sports activities because their body is too fragile. They want to help and are very successful at involving their schools, sports clubs and friends to support Care4BrittleBones.



More information about the ambassadors and their stories can be found on the Care4BrittleBones website www.care4brittlebones.org



5. | PATIENT ORGANISATIONS

We work closely with various patient organisations in all of our activities. We believe that an active role for people with OI is an important aspect of a meaningful and impactful research agenda. Their willingness to advocate for research, articulate their needs and participate in research makes a major difference. We also maintain regular connections with OIFE (OI Federation Europe) and the OIF (OI Foundation, active in the United States and working closely with the Canadian COIS).

We are proud to be informally associated with the following Patient organisations:

- Belgium: ZOI
- France: AOI
- Germany: DOIG
- Italy: AS.It.OI
- Switzerland: SVOI/ASOI
- The Netherlands: VOI

Patient organisations that are interested in working with us are encouraged to get in touch via email dagmar.mekking@care4brittlebones.org.

Overview Care4BrittleBones

key documents

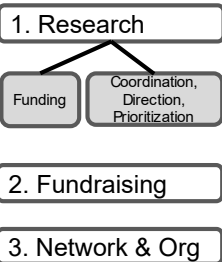
PARTNERS IN RESEARCH

- ERN (European Reference Network/EU)
- BBDC (BrittleBones Disease Consortium / USA + Canada)
- Pharmaceutical Industry
- 800 Researchers
- High caliber international Advisory Board of Scientists

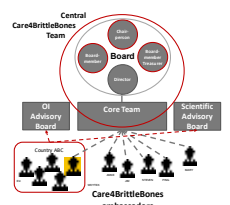
Doc: OIKOS



WHAT WE DO



WHO WE ARE



Better
Care & Cure
through
RESEARCH



HOW WE WORK

Values

- Positive
- Inspirational
- Innovative

We work according to the highest standards as a charity. We are recognized as ANBI, members of Goede Doelen Nederland and certified by CBF

Communications

- Website, e-mail, facebook, twitter
- Phone, teleconference
- Newsletter
- Annual Report
- You Tube

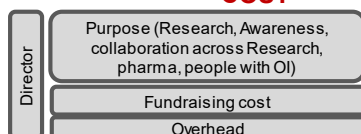
Doc: Case for Support

Doc: Comms Strategy

PARTNERS IN OI COMMUNITY

- Network-Partners:
 - AOI (France)
 - ASITOI (Italy)
 - DOIG (Germany)
 - SVOI (Switzerland)
 - VOI (Netherlands)
 - ZOI (Belgium)
- OIFE (European Federation Osteogenesis Imperfecta)
- OIF (USA/Canadian Patient organization)
- People with OI & their family and friends worldwide
- High caliber international Advisory Board of People with OI (Patient-Experts)

COST



Doc: Accounting (CBF approved standards)

LONG TERM APPROACH

Doc: Multi year plan 2018-2020

- Research
- Fundraising
- Networks
- Operations
- Fundamentals



STRATEGY | AND WAYS OF WORKING

1. | STATUTORY GOAL

The vision of our foundation as reflected in our statutes is unchanged. The foundation aims to promote scientific research into Osteogenesis Imperfecta with the intention of improving the quality of life of people with OI.

The Foundation seeks to achieve its objectives by:

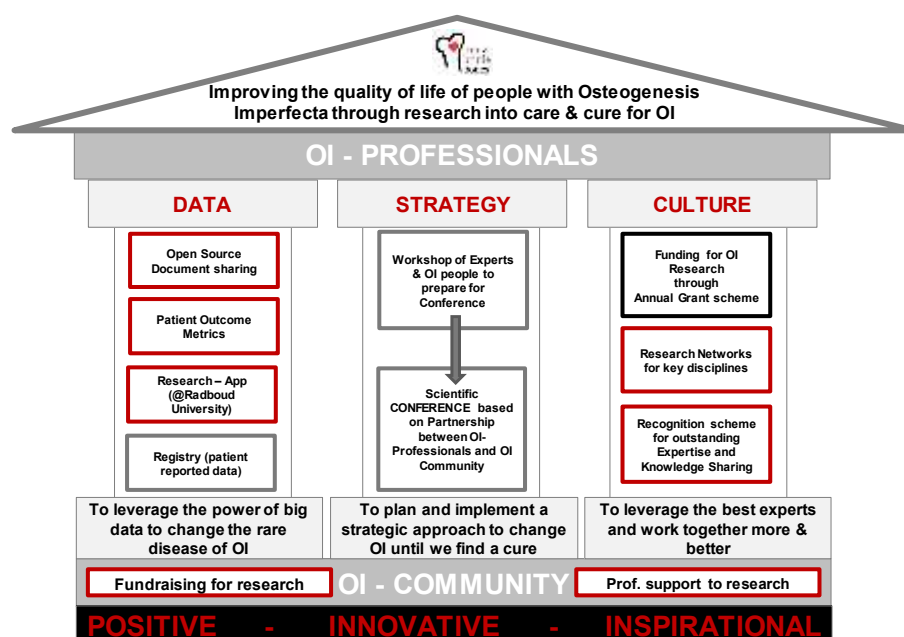
1. Raising funds;
2. Funding research and activities enabling research;
3. Contributing to impactful research by developing priorities and strategies for improving Osteogenesis Imperfecta, supporting collaboration and knowledge sharing regarding Osteogenesis Imperfecta and providing support to research in the widest sense;
4. Informing stakeholders.

We have decided to emphasize our active role in research. As opposed to many other foundations, we are not only a fundraising organisation but also a coordinator, strategic partner and thought leader in research for OI.

2. | STRATEGY

We believe that a two-pronged approach is needed to improve the quality of life of people with OI. Our approach focuses on improving quality of life through short- and medium-term research, related to clinical interventions close to the patient (e.g. improved orthopaedic approach, physical rehabilitation, understanding scoliosis, etc.) and leveraging potential game-changing technologies, which may provide a cure for OI.

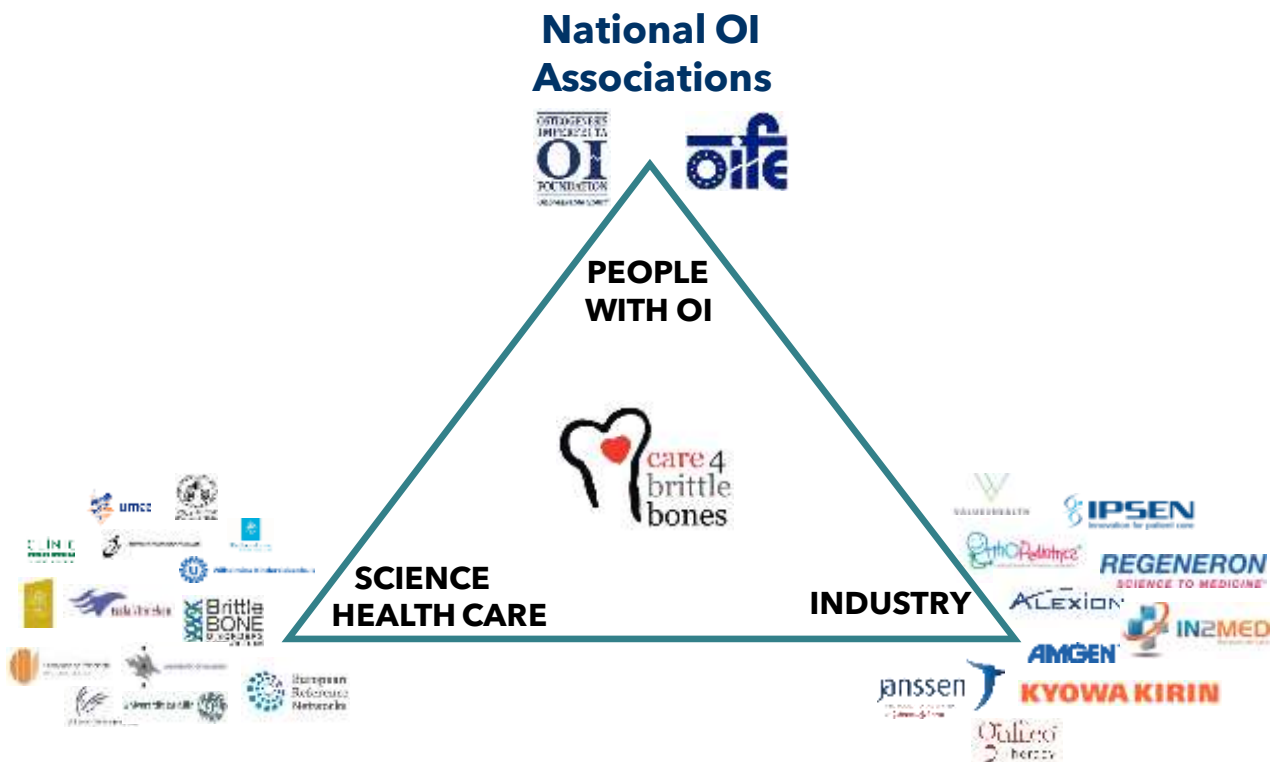
In 2019 Care4BrittleBones has continued to build on its research strategy, which is called "OIKOS". OIKOS is a holistic approach to driving change in research and improving the quality of life of people with OI. The three most important levers and associated strategic actions are: data, strategy and culture.





Care4BrittleBones strives to be a ‘bridge builder’ to connect the three main groups active in the field of OI Research: the OI patient community, healthcare providers/researchers and (pharmaceutical and other) industry. The “QualityofLife4OI” Conference exemplified this aspiration.

We actively work together with all of these communities, providing professional experience, tools and bandwidth to accelerate OI research projects and strategies with a clear link to the quality of life of people with OI in the short-, mid- and long term. Our aim is to contribute to the development of an ecosystem that continuously drives positive change for OI. We realise that we must reach out beyond OI networks to support related rare bone diseases where we can. Bone metabolism still has many ‘blind spots’ that are unknown territory for researchers worldwide. Therefore, supporting the research of one rare bone disease can potentially provide insight into others such as OI. This is important for all of us.



Logos displayed are based on our current stakeholder relationships (2019).

Our current strategy is described in the multiyear plan (2018 – 2020), which has been published online. This document covers the areas of Research (OIKOS), Fundraising, Networks & Reach, Communications and Fundamentals. Each of the areas is underpinned by a more detailed document, defining ways of working to further long-term ambitions and operations. The operational delivery against the plan is described in the next chapter.



3. | WAYS OF WORKING (COMPLIANCE AND TRANSPARENCY)

Foundation Care4BrittleBones is recognised as a charitable organisation in the Netherlands (**ANBI** status, Algemene Nut Beoogende Instelling).

As a charitable organisation, we are firmly committed to the highest standards of operation. This relates to every single process, but in particular to **compliance** with all applicable rules and procedures (including data privacy) and **transparency** (e.g. time writing of the CEO/directeur-bestuurder, regularly reviewing our performance against the goals and sharing our reflections on strengths and weaknesses with our stakeholders).

To demonstrate our standards and ensure we stay up to date on the latest developments in the charity sector we are a member/accredited by the following organisations:

- **Goede Doelen Nederland** – sector organisation for charities in The Netherlands. Through Goede Doelen Nederland we are participating in discussions with other stakeholders in the sector regarding topics relevant to all charities in the Netherlands. As a member, we are aligning with their standards and recommendations.
- **CBF** – Centraal Beheer Fondsenwerving. CBF will annually review all key processes of a foundation and carry out testing to decide if they are robust with regards to governance standards and transparency. This provides extra assurance to our donors that we are operating according with strong internal standards.

In 2018 and 2019 we have implemented the **EU-General Data Privacy Guideline** in a timely fashion. We used the opportunity to virtually review all processes in relation to data and enhanced our data handling methods and tools. All relevant information including a complaints procedure has been published on our website and the changes have been implemented.

Key to our work are the values we have adopted when setting up Care4BrittleBones in 2012: three simple words guide everything we do: **positive, inspiring and innovative**. This applies to our research, fundraising activities and to the way we interact with each other and our stakeholders.





| FINANCIAL COMPENSATION

In 2019, none of the board members, core team or volunteers, ambassadors and advisory board members received financial or other compensation except for the director and one person in a so called “OI-talent role”.






- The talent role was introduced in June 2018 as the foundation wanted to deeply engage people with OI in the way we work “every day”. The role was recruited for through advertisement. The selected candidate provides support to the CEO/director in line with his/her own ambitions and talents. The talent candidate receives a compensation for voluntary work permitted in the Netherlands (€1500). The duration for the role is 1 year.
- The director’s remuneration is explained in more detail in the financial report.





OPERATIONAL DELIVERY | 2019

2019 was a year of growth in all aspects: in variety of activities, involvement of the community and impact on research and quality of life. After a year of consolidation and solid delivery in 2018, 2019 was a year where we stretched ourselves to the absolute maximum to achieve as much as we could together with our network. By doing so, thanks to our strong operational processes, highly motivated volunteers and dedicated professional network of experts, we have exceeded our expectations.

1. RESEARCH 	<ul style="list-style-type: none">• Data:<ul style="list-style-type: none">• Blockchain pilot• Strategic use of Researcher Database• Strategy: Catalyst for Research priorities<ul style="list-style-type: none">• Conference Quality of Life 4 OI• Key4OI Outcome measures• Guideline for Good clinical care• Psychosocial Project.• Prepare Proposal Round 2020 (kicked off at Conference)
2. COLLABORATION 	<ul style="list-style-type: none">• Researchers: 2 well established networks for knowledge sharing• OI Community: Positive collaboration with<ul style="list-style-type: none">• OIFE & OIF in relation to Conference and other projects.• Ambassadors• OI Advisory Board / Patient Network partners
3. FUNDRAISING 	<ul style="list-style-type: none">• Funding from Public• Funding from Trustfunds• Funding from Companies• Funding from Individuals
4. COMMUNICATION 	<ul style="list-style-type: none">• Focus on basics• Sustainable resourcing• Social Media impact
5. OPERATIONS 	<ul style="list-style-type: none">• Further improve project expenditure and sponsoring monitoring• Operational excellence in data management / document mgt (dropbox)

| RESEARCH

- Overall:
2019 has been the best year so far of our foundation in relation to research. Key research projects were completed and several complex projects led or supported by Care4BrittleBones have made good progress. In addition, the Conference QualityofLife4OI was organised and provided a rich forum for exchange under the motto of “stronger by working together”. This has led to a stronger “ecosystem” for OI research overall.
- Research projects status:
Foundation Care4BrittleBones has been involved in 10 research related activities in 2019. Our role varies per project:
 - The **Vitamin K** project of the UMC Utrecht continued in 2019 and is expected to report out in 2020. It looks into whether children with OI have a Vitamin K deficiency. Similarly, the **Eat, Breathe, Sleep** project was started and will be completed by principle Investigator Antonella Lo Mauro in 2020. Other projects supported by us in 2019 include the **OI Variant database project** of Dr. R. Dalgleish and the **Blockchain project**, both will mainly take place in 2020. We have supported the research of Claire Hill in relation to the international validation of the **Quality of Life questionnaire** she developed in 2016. We supported work of Nick



Bishop in relation to the **Research priorities and practices** in different countries.



- 4 projects can be considered as main achievements in support of quality of life and deserve to be mentioned specifically:
 - (1) The project “**psychosocial care for OI**” has been concluded. It was led by Foundation Care4BrittleBones and has already been described in the annual report 2018. The project has delivered and communicated results in November 2019. The findings have influenced the thinking about OI as a “purely physical disorder” and have resulted in various long-term changes in healthcare, at the level of patient organisation and OI-community support and in research. A network of experts has been formed to continue to build expertise in this area.
 - (2) The very innovative project “**Towards a therapy for OI**” of Fleur van Dijk / UMC Groningen, looking into the use of induced pluripotent stemcells for OI has closed out the laboratory phase with very interesting results. More information is available in the next chapter.
 - (3) The most complex challenge has been the development of the **Key4OI** Outcome measurement set in 2018 and 2019, more information in the next chapter.
 - (4) Last but not least, the **Conference QualityofLife4OI**, was organised in 2019. A dedicated chapter in the annual report describes the conference in more detail.
- Our involvement in one research project unexpectedly changed in 2019. The project is “Guideline for good clinical care for OI” led by ERN-BOND. ERN-BOND received new information from the EU in 2019 which means that the development of the guideline cannot be funded by a charitable foundation but only by EU itself. ERN-BOND plans to develop the guideline by 2022/2023 according to a new project plan with slightly different scope. As a result the original project in collaboration with Care4BrittleBones was ended.
- In 2019 we have not conducted an annual proposal round as in previous years as we focussed our financial resources on the organisation of the conference QualityofLife4OI
- We actively managed our research contracts. The status of all running contracts is part of a monthly review in the Research & Fundraising team meeting.
- We were highly involved in delivering research projects ourselves. Given the strong project management capability in the foundation, our involvement has helped to ensure delivery on time and specifications.
- We increased our communication about research significantly by introducing research briefs (summaries of projects that have been written using lay language) and webinars. These materials have been well received by researchers and the OI community.
- We spent EUR 522.800 on our statutory goals: research, information sharing and collaboration for knowledge exchange (incl conference). This is an increase from 2018 and significantly above the initial 2019 budget. The higher spend on goals was enabled by a higher income generated during 2019.



Improvement opportunities, risks and uncertainties in this area:

We continue to work proactively with researchers to start projects on time and keep the projects on track. It is also necessary to maintain or further enhance our communication about research funding capabilities. Thanks to the Conference QualityofLife4OI and Key4OI, knowledge sharing and collaboration have improved significantly in 2019. We believe that these changes are part of a “culture change”. We will continue to reinforce these themes in the next few years in order to anchor them firmly into our ways of working and make a tangible difference to quality of life for people with OI.

| FUNDRAISING

- a. **Overview:** In 2019 we have continued to build our relationships with donors, trust funds and other funding institutions laid in previous years. We also have seen a significant difference in the 2019 fundraising compared to previous years based on two important developments: 1. We have done a lot of fundraising in relation to the Conference. Also we have continued to build our reputation as a health care innovator with Zorginstituut Nederland, which has led to the second substantial grant from them, this time in support of our project Key4OI. These 2 influences have increased the fundraising success and have changed the mix of fundraising sources.
- b. **Conference related fundraising:** In order to raise funds for the conference QualityofLife4OI, we partnered with various companies. We had not received any funding from any pharmaceutical or medical company before and did not have any established relationships in this sector yet. Our strategy was to approach them with professionalism and to provide them with a true partnership experience. We listened carefully to their needs and ensured we were set up to deliver a conference experience that was “at least on par” with similar events organised by professional providers. In addition, we created a differentiating offer due to our strong link to leading experts for rare bone diseases as well as people with OI and other rare bone diseases.
- c. **Fundraising event:** 12 fundraisers were organised this year: A sponsored run in the area of Munich and a concert in the prestigious Elbphilharmonie of Hamburg, the Dam tot Dam walk & wheel and the “Spijkerbroekengala” were amongst the most successful events this year.



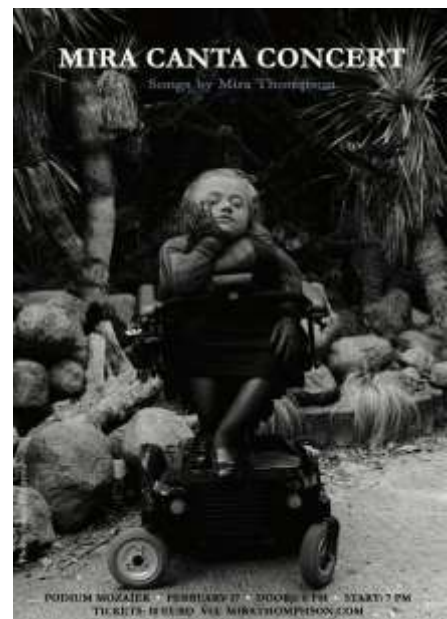


International contributions: Patient organisations from other countries contributed strongly to various research projects coordinated by Care4BrittleBones and participated actively in the Conference. Other contributions (e.g. financial contributions) were limited, except from 2 events in Germany. Many patient organisations in other countries struggle to provide their members with the most basic support and are simply not in a position to contribute significantly to research funding. The main contribution in 2019 came from OIF and was related to the Conference QualityofLife4OI.

- d. **Crowdfunding:** In 2019 we continued to experiment with crowdfunding. Several people with OI surprised themselves with the funding they were able to raise with the support of our highly professional Kentaa platform, which we established in 2018 (www.research4oi.org). Also the donation option on facebook is increasingly popular when OI community members are celebrating a birthday or another festive occasion.

We continue to believe in crowdfunding for Care4BrittleBones. It is a perfect way to educate about OI and make the case for change in combination with supporting research. It makes use of and develops the creativity, skills and networks of people with OI and therefore is critical for the “soul” of our organisation. We raised ca. 20.000 through crowdfunding (research4oi.org, geef.nl, facebook mainly).

- e. **Fundraising education:** To strengthen the fundraising skills of people in our network we held a webinar which was well attended. The webinar is available on [Youtube](https://www.youtube.com/watch?v=...). We expect the Core team of Care4BrittleBones to keep educating themselves in raising funds. There is just no better way to learn about it than actually doing it. It also helps the core team to be aware of the challenges and the satisfaction that it provides.
- f. **Long term donors:** Most donations are “one-off”. There is a need to increase the long-term donor base which has greater potential. We had the great pleasure inviting all our long term donors to a concert of the jazz singer Mira Thompson in Amsterdam in February 2019.



Improvement opportunities, risks and uncertainties in this area:

Research for OI requires a long term strategic approach. Most of our projects take 2 years or longer. To make the impact on quality of life we will need to improve our long term funding, which is not sustainable at the moment. Also, we need to focus on internationalising the fundraising income in collaboration with the OI community in other countries to address the sustainability challenge. We believe that more is possible in this area, not taking anything away from the patient organisations, but rather building their fundraising skills, which they can then also use for themselves.



| NETWORK AND REACH

Core team: Our organisation model has evolved further in 2019. 3 core team members were replaced by new volunteers, which provided a fresh perspective and new energy for our foundation. Various other volunteers joined subteams and specific activities and were critical to deliver on our objectives. Each volunteer in the core team has strong professional acumen and expertise. The Core team meets on a monthly basis to discuss strategic topics relevant to everyone. Meetings are held in English. In addition, various sub-teams exist that handle the operational delivery. A robust infrastructure of collaboration tools are enabling us to work seamlessly together.

In memoriam: In all activities of foundation Care4BrittleBones people with OI have an important role. Nobody knows more about OI than people with OI themselves! Sadly, in 2019 we have lost 2 wonderful friends with OI. Both of them have passed away unexpectedly, far too early and have left a big hole in our hearts, as they played such a crucial role.

- One of them was **John van Hal**. He newly joined us in 2019 with great enthusiasm, strategic insights and true leadership. In a very short time he started to lead projects and teams and we had plans to take him on in a formal role in the foundation. His involvement during the Conference and Key4OI have been his key contributions.
- Also, the OI community is grieving the loss of **Ida Männistö** from Finland. She was a board member of the European OI Federation (OIFE) and involved in the steering committee of the Conference QualityofLife4OI and Key4OI.

We are missing them greatly and are thinking back with huge gratitude on their help with the foundation Care4BrittleBones.

Ambassadors: Ambassadors are people who want to help us improve the impact on quality of life through research. They are our eyes and ears to spot opportunities for fundraising and are often actively engaged in research or research support. Many of our ambassadors played a key role at the Conference QualityofLife4OI, moderating workshops, taking pictures, recording podcasts and vlogs etc. In 2019 the number of ambassadors supporting Care4BrittleBones has increased again. We are particularly pleased with new ambassadors in countries outside the Netherlands who have joined us.





As a whole, people in the international OI community are becoming more aware about our contribution to OI research.

- a. **Engagement:** Driven by the needs of the Conference we have pulled our engagement levels with researchers, healthcare providers and the worldwide community to a new level. We have grown the number of people who receive our newsletter, the number of posts on social media has increased tremendously, underpinned by a very active and strategic communications team lead by our new Communications board member. The response from the community is very positive. We now have a database with 1399 researchers, people in the OI-community and donors.
- b. **Website:** the website, which was refreshed at the end of 2018, was kept up to date in 2019. It provides comprehensive and user-friendly access to a wide range of resources which help professionals and people with OI to have a better quality of life.
- c. **Webinars / videoconferences:** As our research projects involve researchers and people with OI from around the world, our tools are also fit for this purpose. In 2019 we held several worldwide videoconferences and webinars to educate, learn together and work together on various topics relevant to quality of life.

Improvement opportunities, risks and uncertainties in this area:

The main operational concern in 2018, as stated in the last annual report, was the area of communications. In 2019 this area has transformed completely into one of the most buzzing and value-adding activities of Care4BrittleBones thanks to our new board member Celine Disch. We are keen to maintain this impact and will seek to bring in some additional resources for the long run.





| CONFERENCE “QUALITY OF LIFE 4 OI”

The highlight of 2019 was clearly the conference QualityofLife4OI, which took place from 22 – 25 November 2019 in Amsterdam. It has been a huge success and exceeded our expectations in many ways.

Key numbers:



Programme highlights and key discussions

The conference objectives were to:

1. Create focus on health-related quality of life for OI
2. Promote use of patient outcome measures (PROMs and CROMs, in particular Key4OI-measures)
3. Create a shared view of standards for good clinical care for OI which drive these outcomes, including the standard of multidisciplinary treatment.
4. Provide insights on cutting edge research in priority areas for people with OI
5. Promote learning and collaboration within the communities involved

These objectives were all fully met. The strong feedback in the final evaluation by the participants has confirmed that. Without your support this conference would not have been possible.

Let us give you a few examples of what we achieved, how it was achieved and why it matters.



1. Improved collaboration on various levels

The conference enabled us to make great progress in relation to conference tag line: “Stronger by working together”.

Working together across rare diseases and across disciplines

Many international networks used the conference to meet, e.g. the ERN-BOND held a meeting in Amsterdam, a rare bone meeting took place bringing together researchers, industry and patient organisations, various project teams and research teams met in Amsterdam before and after the Conference itself. Various projects actually worked towards the conference in order to receive feedback from the different stakeholders. The interactive character of the programme helped to bring people together across disciplines and across researchers and healthcare providers. One of the key themes was multidisciplinary work.



A strong voice of patients

By design the conference integrated patients as equal partners on all levels. They were part of every decision, every team including the programme committee, every panel and 20% of the speakers were expert patients.



This really changed the dialogue and made the conference even more impactful than an event with only healthcare providers and researchers. This was confirmed in the feedback from the participants. Various patients advocated and were role models for patient advocacy and patient participation in research. They shared their passion for research and why they believe patient participation is so important. The dialogue was more focused on what is important to patients than other conferences. 30% of the conference participants (104 individual people) had a patient background (either patient or family of someone with OI/rare disease).






The conference had an optional patient session before the official opening session to encourage networking and peer support. One of the objectives of this session was to encourage effective patient participation – the do’s and don’t of participating in a professional scientific conference. This advice was perceived as very helpful. Most workshops started with a presentation about the “patient view” on the workshop topic. This presentation was not just an individual person’s view but the outcome of a longer dialogue and an OI Sounding Board teleconference which took place one month before the conference to which literally “everyone with OI everywhere in the world” was invited. A powerful way to connect the community and jointly prepare for the conference and increase its impact!



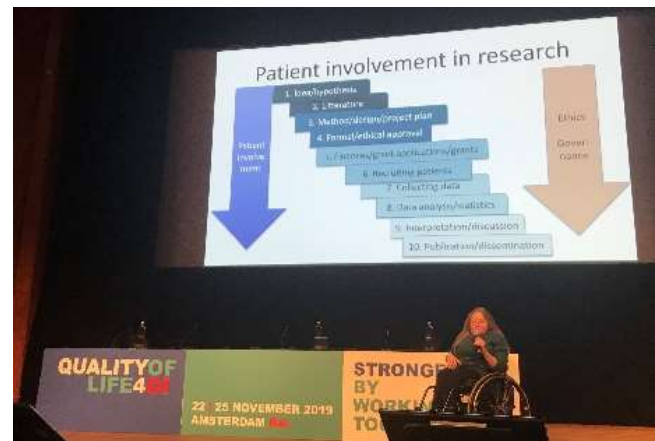
One of the innovative ways the conference encouraged focusing on real patient concerns was a session in which multidisciplinary teams from around the world discussed how to treat OI using “OI-personas”. These OI-personas were not real people but representative personas created by people with OI for the purpose of this conference with attributes and stories that resonated with their own lives. Six personas (children and adults) were created and were instantaneously embraced by everyone. The technique can be used in future conferences too.

Examples:

		
<h3>KATE</h3>	<h3>DWAYNE</h3>	<h3>MIA</h3>
<p>MILD OI A curious girl, timid and quiet growing up with very protective parents</p> <p>Fully mobile Age 8 Junior school 1 brother (without OI)</p> <p><i>Hobbies:</i> Harry Potter books, Disney</p>	<p>MODERATE OI Laid back cool boy mostly at home</p> <p>Uses wheelchair most of the time but can walk small distances Age 15 High school 1 brother (with OI)</p> <p><i>Hobbies:</i> Playing guitar, social media, online gaming and</p>	<p>SEVERE OI Strong willed, eager learner with very supportive family and high aspirations</p> <p>Using wheelchair Age 17 Specialist school, only child</p> <p><i>Hobbies:</i> Reading, arts and crafts, non</p>



Last but not least a highlight of the conference were several presentations and workshops in relation to patient participation in research. The goal of these sessions was to educate and encourage the conference participants to make use of the expertise of patients at all levels of the research & development cycle. A patient empowerment workshop provided a forum for exchanging best practices in this area. The use of real examples of effective patient engagement was helpful to address questions and concerns.



Industry as an important partner to achieve better quality of life for OI and other rare diseases

The conference received strong support from a broad range of pharmaceutical companies and other industry. They were positioned as a critical part of the “ecosystem” providing better solutions for quality of life of people with OI and other rare bone diseases.

They engaged with researchers, healthcare providers and patients in various workshops and panels. They also played a vital role as sponsors of the conference. A centrally located exhibition space provided opportunities to engage with the sponsors and exhibitors throughout the entire conference.

PLATINUM SPONSOR

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OTHER SUPPORTERS





2. Rich scientific dialogue across multidisciplinary experts worldwide

The Conference provided a platform to share experiences and innovative insight into research. More than 60 international speakers reported on their work and several projects actually worked for months towards reporting at the Conference, especially the Key4OI-outcome measurement project and the Roadmap for OI Surgery-project. 5 deep dive sessions (medication, surgery & rehabilitation, psychosocial, diagnosis and patient empowerment) discussed the latest thinking on “standard of good clinical care” in these areas.



One of the highlights was the report of the newly developed Key4OI Outcome Measurement set. It was the first time it was shared with a broader audience. The idea of the project was embraced by the conference participants and many countries immediately opted to work with it in the future. It marked the start of healthcare providers from 17 (!) countries volunteering to pilot the Key4OI Outcome Measurement Set. They continue to meet and work together in 2020 and 2021 to implement the common dataset. Key4OI was co-created in 2018 and 2019 by healthcare providers and patients with OI from 16 different countries around the world. It aspires to be a pathfinder for other rare diseases. Most certainly is provided a strong boost for collaboration and healthcare improvement in OI. Foundation Care4BrittleBones as the owner of the concept will explore with the EMA how Key4OI could be used in clinical trials going forward.

3. Other highlights worth mentioning:

- The conference made use of both **traditional communication** methods (posters, orals, abstract books, panels etc) as well as **novel technology and approaches** (e.g. commenting and voting online / realtime, large visual displays, interactive theater, guided poster walks for patients etc.)
- **New Investigator Mentoring scheme:** The conference offered a coaching scheme between experienced and new investigators. During the Conference registration all participants were asked if they would like to mentor new investigators or if they would like to be mentored. This led to ca. 80 coaching relationships which were established at the beginning of the conference. Many of them will extend well beyond the conference. An optional format for structuring the coaching relationship was provided upon registration.
- The **conference dinner** was an inspirational highlight with several music performances from people with OI. It even led to some healthcare providers dancing on the table! The performances were delivered by the international rapper and inspirational speaker Sparsh Shah (16 years old!) and a Jazz Singer from Amsterdam, Mira Thompson.
- **Learning Journal:** We provided a learning journal to capture the key lessons at the end of each day and bringing them together to a powerful plan of action on the last day.





- **Awards:**

Several awards were provided to celebrate outstanding achievements.

- 2 awards for lifetime contributions to quality of life for people with OI awarded to Kathleen Montpetit (Canada) and Julia Piniella (Spain)
- 6 young investigators awards
- 3 awards for best abstracts
- 1 conference choice poster award

- **People with OI in action** supporting the conference (60 OI volunteers in total):

Many people with OI were active as speakers and co-organisers of the conference. In addition, 20 full time dedicated people with OI took on roles as photographer, workshop logistics managers, journalists, moderators, receptionists, performers, vloggers etc. The conference was actually showcasing and developing people with OI to use their professional talents. This had a lasting impact on them as individuals. Quote of Miranda van Rooijen on facebook shortly after the conference “...This conference has really changed my life. I has completely turned around how I think about my doctors and research”). It has also changed the perception of many professional participants in relation to the contribution patients can make in research and healthcare (see also feedback / evaluation). Whilst some of them had wondered, if this would work, the overwhelming majority found the participation of patients value adding.



Results / Outcomes

- Overall we have provided a strong example of a conference organized together **with patients as partners**. The conference:
 - had full support from industry. Obviously all rules and regulations governing good conduct between industry and patients and other people not authorized to prescribe drugs were observed .
 - provided an intensive and relevant learning experience for the different types of participants. Healthcare providers received CME accreditation points for attending the event.
 - integrated patients on all levels of decision making, as speakers and as conference co-organisers.
 - offered (thanks for our generous sponsors and many volunteers!) perfect conditions for people with OI to participate. Some examples of the support provided:
 - solutions for people with hearing impairment
 - furniture to relax physically during the long conference days
 - access to the stages for wheelchair users in various locations, mobility solutions for in total 50 (!) participants in wheelchairs, who have been able to join all parts of the programme.
 - support to OI-patients throughout their entire stay including a special helpline in case of any incidents (which luckily did not occur!)

It was the very first time that a conference was organized with such a significant role of patients with rare bone diseases. The impression was that it was the start of a culture change that will lead to sustainable value for patients with OI in the long run.

- The conference had a rich **conference programme** geared to support quality of life for OI and other rare bone diseases. 65 international speakers contributed to it. It



provided a comprehensive overview of the latest thinking about multidisciplinary care for OI and other rare bone diseases. It enhanced the awareness about the relevance of outcome measures and true focus on the needs of the individual patients with OI. All participants also were energized by the promise of future big data and comparable data becoming available in the next few years through Key4OI.

- The conference was delivered **on plan and on budget**. 340 participants were present during the conference, of which 30% of people were from the patient community (OI and other rare diseases). The project plan has been updated with learnings based on the feedback of everyone involved.
- About **60 people with OI** were highly actively engaged as speakers and co-organisers of the Conference. In total 104 people with OI and other rare bone diseases participated (ca. 30%).
- 1 [aftermovie](#), 9 [podcasts](#), 3 [vlogs](#) (1, 2 and 3) and 6 [video interviews](#) were recorded
- A [conference website](#) with slide presentations of speakers has been created and remains live
- A [comprehensive abstract book](#) including all abstracts received has been issued (paper and online).
- Countless Tweets and other **social media** posts in relation to #qualityoflife4oi have been posted
- About 1000 **photos** were taken and many used in social media.
- Coverage about the conference in various media channels like TV ([jeugdjournaal](#)) and [Seven Days](#).
- A **conference social programme** with a visit of the Amsterdam Canals by boat, a conference dinner with music performance and a cheese and wine reception.
- 3-5 **publications** are expected to be published in 2020 due to projects related and initiated by the conference. All of them have a strong voice of patients included.
- A beautiful **piece of art work co-created by the conference participants**:





Evaluation / Feedback from the conference participants

All conference participants were invited to provide feedback within 1 week after the conference. We received 88 responses (25% of all respondents). The evaluation results was very positive. The average satisfaction rate of the participants was 8.3 (out of 10) with similar results across patient community, industry, healthcare providers and researchers. The feedback on the organization of the conference (catering, logistics management, registration, support etc) has been enthusiastic. Also the participants have confirmed that the learnings obtained mattered to their professional practice and that they would be keen to join another conference like this one. There was a lot of support for having people with OI and rare bone diseases as discussion partners during the conference.

Question(n=90)	Negative	Neutral	Positive
Effectiveness of the discussion with people with OI/rare bone diseases in the room	5%	12%	83%
Feedback on organisation and management of the conference	2%	7%	91%
Able to use learnings in practice	5,5%	na	94,6%
Join another conference like this	8%	na	92%
Communications	1%	7%	92%

CZ Award 2019

On 28 November 2019, just a few days after the conference, we have received the CZ Award for a pitch about the most impactful project for patient quality of life.





IT | & SYSTEMS

Foundation Care4BrittleBones believes in innovation and technology. We therefore need to lead by example, ensuring we work to the highest standards with regards to our data and systems. Many important upgrades and updates had already taken place in 2018. In 2019, we deepened the initial implementations and made additional improvements, partly triggered by research projects or the Conference:

- **e-bookkeeping tool** (implemented in 2018): Implemented more extensively in 2019
- **Dropbox** (implemented in 2018): Expanded the use of this web-based document sharing system
- **New website Care4BrittleBones** A completely new website was implemented at the end of 2018. In 2019, the website was updated and improved
- **New website Conference QualityofLife4OI** With thanks to 'AMINNOVATION' who supported us, we were able to develop and run a strong Conference website in support of the conference, facilitating extensive knowledge transfer among all participants
- **Policies and procedures:** We developed a data management plan in line with the new GDPR rules introduced in 2019, published our approach on data privacy and implemented a complaints policy and procedure. No complaints have been raised through this policy in 2019.
- **Delphi Survey:** We conducted several anonymous Delphi surveys, facilitated by ourselves, supported by a user friendly web based tool
- **Mailchimp:** Our newsletter provider has been reviewed for GDPR compliance and upgraded to a commercial, paid account due to the size of our audience
- We are working with various **social media channels and other platforms such as YouTube and SoundCloud, where we have dedicated channels** (see also the chapter on communications)
- **Crowdfunding platform Research4OI** continued to run in 2019. Whilst it works technically well, additional effort is required to increase its use.





MAKING A DIFFERENCE | THROUGH RESEARCH

1. | APPROACH: COLLABORATION AND INNOVATION

2019 has been the best year of our foundation thus far in relation to research, delivering on our long-term research strategy defined in 2017. The aim of this research strategy is to make a sustainable difference to quality of life for people with OI. The mission of the Care4BrittleBones foundation is ***“to provide and leverage resources and capabilities that enable the OI ecosystem (e.g. researchers, healthcare providers, industry, people with OI) to deliver impactful research that improves quality of life for people with OI.”***

We deliver on our mission by funding research, by leading and/or supporting research with our own resources and/or by enabling an environment in which knowledge exchange can thrive. All research ideas need to make sense for the OI-community, which is a vocal, tightly networked, engaged and innovation-minded community. A great environment for meaningful clinical and pre-clinical research!



2. | RESEARCH HIGHLIGHTS

Of the 10 research projects which we were involved in or led in 2019, we will highlight only 2 in this part of the annual report: one focused on healthcare improvement (project Key4OI) and the other one on basic science (project “Towards a therapy for OI”). Foundation Care4BrittleBones spends 50% of its funding on healthcare improvement and better treatment of OI. The other 50% is to support projects from basic science, important for long-term improvements of quality of life. Care4BrittleBones believes that it is important to focus on both progress in clinical care and progress in research about the causes of OI, in order to learn whether the OI related symptoms can be diminished or even reversed.

1. Key4OI – creating a core outcome set for multidisciplinary care and research |

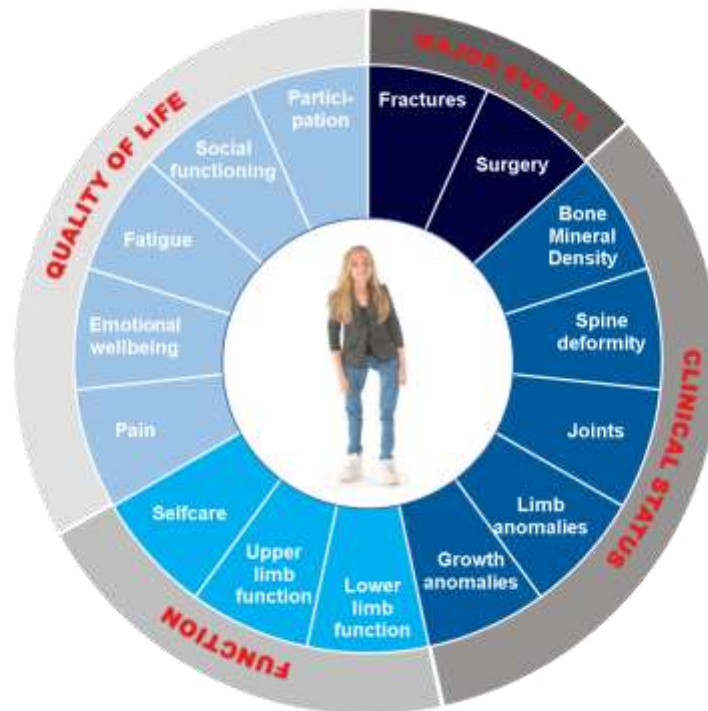
Projectteam: W. Nijhuis, A. Franken, G. Janus, R. Sakkers, M. Verhoef

Expert team: K. Ayers, Ch. Damas, L. Folkestad, A. Forlino, P. Fraschini, C. Hill, R. Kruse, L. Lande-Wekre, L. Michiels, K. Montpetit, L. Panzeri (S. Paveri), F. Rauch, JP Salles (V. Porquet-Bordes), O. Semler, J. Sun, M. To (Eric Yeung), L. Tosi, L. Zhytnik, C. Zillikens,



Project management: Dagmar Mekking, CEO Care4BrittleBones

To date, a complete understanding of how the disease and related health problems impact the patients' quality of life, both for children and adults, is still missing. There are no harmonized, reliable tools for the proper assessment of OI patients. In addition, there are no derived outcomes available at an international level. From a clinician's perspective, a standardised approach to evaluation of OI that captures the major impacts on patient outcomes is lacking, leading to variability in the breadth, depth and types of tools used between and within countries. Most of all, the integration of patients' perception of quality of life is often absent.



The primary goal was to define the minimum set of measures for OI, focused on patient outcome. Patient reported quality of life and medical data (clinical information, imaging, genetic evaluations, etc.) will be the key for the implementation of health care improvements and better therapies focusing on quality of life of people with OI.

This goal is underpinned by various projects:

- The development of a content valid international approach to measure QoL in children with OI – led by Dr. C. Hill, Sheffield/UK.
- The development of a core outcome set for children - led by Dr. R. Sakkers, Dr. W. Nijhuis and Dr. M. Verhoef, UMC Utrecht / NL.
- The development of a core outcome set for adults – led by Dr. A. Franken and Dr. G. Janus, Isala/NL.
- The development of research priorities from a patient's point of view and what patients are willing to "contribute" – led by Prof. N. Bishop, Sheffield/UK.
- Key4OI compass; use of outcome information to decide together in curative care chains.



Care for the patient with Osteogenesis Imperfecta is complex. There is a critical need to better define outcomes, particularly those that matter most to patients and their families and to standardize the methods by which these outcomes will be measured.

The underlying research questions for project Key4OI are: “What outcomes should a multidisciplinary OI team track at each stage of treatment, reflecting what matters most to people with OI throughout their lifetime? How could this data be collected, evaluated and compared taking into account both patient reported outcome measures and clinically reported outcome measures to drive sustainable insights for healthcare improvement and research into OI?”

The primary objective was to reach multidisciplinary and international consensus for a standard set of outcomes in OI care and research. To ensure the outcome set is relevant and reflects the view of people with OI, people with OI were involved in all stages of the process. The standard set had to be comprehensive enough to cover all main aspects of OI relevant for treatment and research, yet practical enough for sustainable implementation. This would allow teams around the world to measure their own performance in a consistent fashion.

In November 2019, the Key4OI Core Outcome set has been agreed. It summarizes the recommendations of an international, multidisciplinary working group, based on input from 16 focus groups held around the world. Each focus group was based on a detailed script and took several hours.

The Key4OI outcome set has been presented during the Conference QualityofLife4OI and will be published in the first half of 2020. The journey will continue in 2020/2021 with 6 locations/ countries piloting the approach:

- (1) Shriners Hospitals for Children, Montreal / Canada,
- (2) Alfred I duPont Children’s Hospital, Delaware / USA
- (3) Norway
- (4) HongKong SAR / China
- (5) Aarhus / Denmark
- (6) NL (lead by Isala, Utrecht UMC)

11 other countries have expressed interest in working with Key4OI in the future: Brazil, Israel, Russia, Switzerland, India, UK, Italy, Belgium, France, Germany, Estonia. They are forming the Key4OI Affiliates Group.





Plans longer term

All funding supporting Key4OI has been generated through the OI community and through the generous work of hundreds of hours of many Key4OI experts and people in the project team (see above). The Care4BrittleBones Foundation has been requested to manage the further development of the Key4OI set. The foundation is committed to provide resources to fund future work and provide a platform for learning & development related to Key4OI.

No industry funding has been received. In the next 3-5 years we aim to create a FAIR data environment in which Key4OI data can be aggregated on an anonymous basis to boost healthcare improvement and research into OI.

2. Towards a Therapy for OI – Fleur van Dijk / UMC Groningen |

An example of potentially game changing research funded by foundation Care4BrittleBones is a project that started in 2014 by principle investigator Dr. Fleur van Dijk / UMC Groningen, in the Netherlands. The goal of the project was to repair the genetic mutation in OI and to take steps towards a therapy for OI.

The study started with a few adults with different types of OI donating a skin biopsy and urine sample to the Isala hospital in Zwolle, the Netherlands. These cells went to the iPSC-CRISPR expertise center in the UMCG Groningen. From the cells provided by the patients, stem cells were generated, and the repair was performed using the highly innovative CRISPR-Cas9 method. The study was initially delayed, because the pluripotent stem cells (called iPSCs) generated from the skin biopsy and urine samples were not stable. The project team carried on through this difficult phase and ultimately was able to make repaired, stable iPSC cells from urine cells and skin cells. The laboratory was able to demonstrate that these cells provided a different quality or different quantity of collagen, typical for OI. The project then went into the next phase geared to increase the number of repaired cells. This process step was again very challenging, as the cells were not stable in the first place. They finally succeeded due to their perseverance and the application of an innovative approach for selecting the cells. This also meant that the project could be completed with the final step: testing if the genetic mutation had actually been repaired and if no “off target effects” had been caused anywhere in the genetic material of the iPSC. This test was performed with whole genome sequencing. No off-target effects were identified! The research project has become a flagship project of the University Medical Center of Groningen and especially the iPSC CRISPR-centrum. It is an excellent example of collaboration between people with OI and basic science enabling highly innovative research. The next phase of this research started in 2019.

3. | LONG TERM OVERVIEW OF RESEARCH SUPPORTED BY CARE4BRITTLEBONES (2012 – 2019)

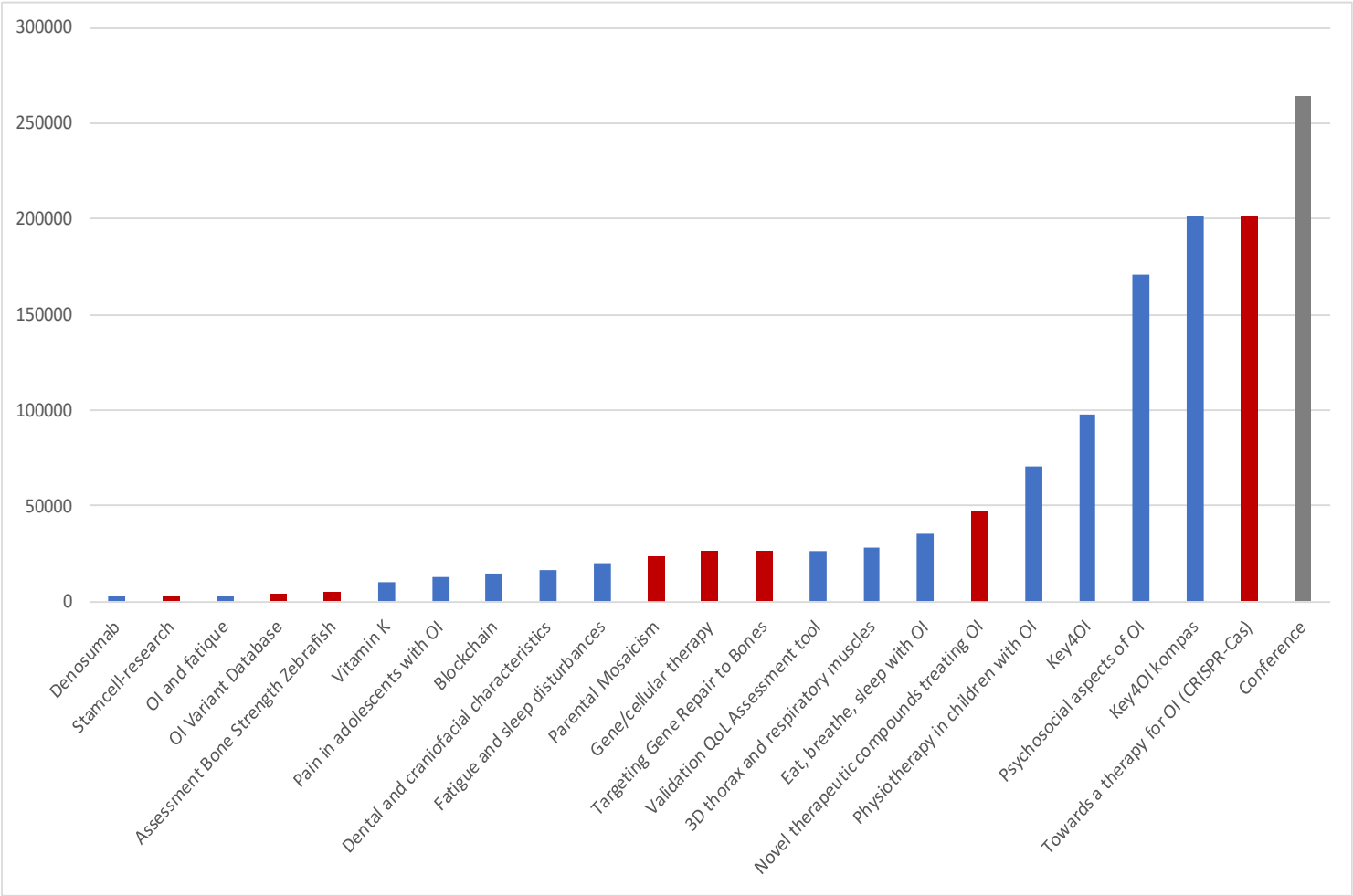
Over the past 8 years Care4BrittleBones has enabled 23 research projects, which are clearly linked to the quality of life of people with OI. These projects are a mix of basic research (focusing on the long term and potential game changing technologies) and clinical research (focusing on areas that bring potentially immediate benefit for people



with OI in the short or mid-term). Many of them are still ongoing, as they often take longer than 2 years to complete due to the complexity.

An overview of the research and research collaboration projects supported from 2012 – 2019 is displayed below.

RED = Basic research. **BLUE** = Clinical research. **GREY** = both





FUNDRAISING FOR OI |

1. | APPROACH: EVERYTHING IS ALLOWED! JUST STAY TRUE TO OUR VALUES...

When starting the charity, we were warned that raising funds is an extremely frustrating business, where a lot of resilience is required to keep going. We experienced it very differently!

We raise funds through events, products and donations in collaboration with our network of highly engaged volunteers. We operate from a concept of 'everything is allowed', as long as the activity is clearly in line with the values of our organisation: **positive, inspiring and innovative**.

We encourage everyone who is fundraising for us to start by considering their special talents first. What do they love to do? What gives them great energy? This simple approach has led to a range of creative and deeply inspiring FUNdraising moments, as well as a high satisfaction and even stronger participation from our wonderful volunteer community, many of them including people with OI and their friends and families.

2. | FUNDRAISING OVERVIEW 2019

The income from individuals including the income from selling tickets for the conference QualityofLife4OI amounted to ca. €154.000. The income from companies, especially from pharmaceutical and other medical companies was strong and amounted to more than €147.000. We had not received any income from that sector before. Income from NGOs and governments amounted to ca. €131.000.

Working with pharmaceutical and other companies in the medical sector

In our multiyear strategy, we described the role we wanted to play in improving quality of life for people with OI: To provide resources and capabilities for the "ecosystem" that can enable a better quality of life for people with OI. This includes researchers, healthcare providers and patient organisations, and also pharmaceutical and other industries. Until the end of 2018, Foundation Care4BrittleBones did not have any partnerships with the latter. We also didn't receive any support from pharmaceutical or other companies in the medical sector. We waited until the circumstances were right to engage with this sector and we were able to do this well. Many board members of foundation Care4BrittleBones have a corporate background, and are acutely aware of the need for compliance and a strong reputation as a "licence to operate".

In 2019 we have learned a lot about the rules governing the interactions between this sector, healthcare providers and patients especially with regard to Conferences. Our guide in this has been the CGR (Stichting Gedragscode Geneesmiddelen Reclame, www.cgr.nl). They are the owners of a Code of Conduct for pharmaceutical advertising in the Netherlands, which is based on the Dutch Medicines Act (Geneesmiddelenwet) and EU Directive 2001/83/EC. The code has been key in establishing our behaviours from the start. We are grateful that the CGR has provided us with practical advice in order to get it "first time right" during the Conference Qualityoflife4OI.

In addition we regularly reviewed our approach within the board of Care4BrittleBones, ensuring all perspectives and long term implications had been taken into account, keeping



our reputation in good shape. These discussions were supported by a stakeholder map with all key pharmaceutical companies and other industry and key stakeholders in these companies. The map includes not only companies relevant for OI, but also companies active in rare bone diseases as we know that our rare bone world is strongly interconnected.

Fundraising events

Fundraising events and generous individual donors also contributed to the fundraising results. From “Walk & Wheels”, to concerts and a gala, from school runs to personal challenges - there were many creative ways in which funds were raised. Here we highlight just some of the many fantastic initiatives that took place in 2019 to raise the much-needed funds for OI research.





Running & rolling for stronger bones

A highlight in our fundraising year is the Dam to Dam run which takes place in September in Amsterdam. This year we were active in two of the activities related to the "Dam tot Dam Loop": We participated with a group of runners in the classic 10 English Mile run from Amsterdam to Zaandam. We also participated in the Walk & Wheel with several people with OI using wheelchairs. Both days were filled with lots of laughter, love and sunshine and a little bit of beer. The result of the hard work: 12.000 Euros were raised! One of the most memorable moments was the surprise from one of our friends participating in the run, announcing that she had found a friend ready to donate 6000 Euros to our foundation as part of a company jubilee! What an amazing heartwarming surprise from our friend, but also this unknown donor, who we are incredibly thankful for!



All the runners who raised funds for Care4BrittleBones do so via www.research4oi.org, one of the leading providers of custom made fundraising websites. Care4BrittleBones has created an account free for anyone to use for fundraising for OI. It is easy!

Go to www.research4oi.org -> Choose your language -> Select "join in now". On this very user-friendly site, runners can set up their personal action page within just a few minutes. Afterwards, they can share the link to their action page by email and social media with their networks, inviting them to support their personal and charity goal. The website is available in English, German or Dutch and is also used by fundraisers for our foundation in other countries.

Want to run with Care4BrittleBones?

Send an e-mail to run@care4brittlebones.org and let us know!





Harry's challenge

Huge thanks go our volunteer Harry, who is one of our most loyal and committed ambassadors to support us. He spent some time in Spain and set himself a challenging target: Climbing the very steep stairway of the mountain Puig of Maria. The mountain was a pilgrim site since the 12th and 13th century and people used to climb it to pray for help to Maria during the times of the plague. The long climb is incredibly hard for someone with OI to do. He worked towards it and made sure he was physically ready. His aim was to raise 375 Euros, one Euro for each of the 375 steps in the staircase on his way to the top. He got help from 3 lovely companions and they made it. They look back with great satisfaction on raising successfully 400 Euros and making an impact on OI research through their very own legs! Thank you Harry, Tamara, Timothy and Pepita!



School run in Germany

Many schools have been active in raising funds for OI research. We really appreciate this as we can combine the fundraising with educating children about OI! One of the most beautiful sceneries for a fundraiser this year surely was the Starnberger See, where Felicia von Huene organised a school run of the Gymnasium Tutzing in support of Foundation Care4BrittleBones. Felicia has been an ambassador for Foundation Care4BrittleBones for many years. She has a niece with OI. For several years she worked towards an agreement with school management to do the school run, which was a huge success: A whopping 2500€ for stronger bones! Thank you very much, Feli!!!





Spijkerbroek Gala

We love to combine fun and fundraising. The highlight in 2019 in that respect was the Spijkerbroek Gala, organised under the inspiring leadership of Esther Herber and her team. Foundation Care4BrittleBones was one of 5 charities who were supported by this hugely successful gala. We received the amazing amount of 32.000 Euros, which goes, in its entirety, to the Expertise center for children with OI in the UMC Utrecht in the Netherlands for their fantastic contribution to the Key4OI project.





COMMUNICATIONS | AND NETWORKS

OI has no borders. For OI, as with many rare diseases, working together collaboratively amongst patients and amongst Health Care Professionals is absolutely essential.

Key reasons why this is so important:

1. **To have a sufficient cohort size (group of patients) to conduct research**
2. **To compare** results of treatment across different countries. No two countries apply the same treatment at the moment.
3. **To enable large, multinational projects effectively, efficiently and with maximum impact**
4. **To ensure the insights gained in one country are applied in other locations** (in general, only 14% of research leads to change in another location after an average of 17 years!)
5. **To cluster funding for maximum impact**
6. **To learn together faster and achieve a greater improvement in quality of life for people with OI with the limited resources that we have. This is true for:**
 - Working together across the OI community
 - Working together amongst OI researchers
 - Working together between OI community and OI researchers

These reasons really apply to any rare disease. For any rare disease working together is “common sense”, but is not yet “common practice”. The research for most rare diseases is largely conducted locally with limited resources. This is the current reality for OI research. Communications and strong networks are important to understand each other, trust each other and learn together. Only by working together and sharing results can we make greater strides in OI research, which in turn will lead to improvements in the quality of life for those in the OI community.





| OI COMMUNITY NETWORKS

In 2019, more than 320 people have been involved as volunteers of Foundation Care4BrittleBones. They are active in raising awareness, in supporting research, in raising funds and -last but not least – running the activities of our foundation. We connect with our volunteers individually in webinars and video conferences. Also, we interact with patient organisations in various countries and work together with them to achieve shared objectives. Our OI-world is a small world. We strongly believe we need to work together to improve quality of life for people with OI.

VOI – Dutch OI Association

Foundation Care4BrittleBones originally started due to a call of the Dutch OI organisation VOI (Vereniging OI). In 2011 the VOI urged its members to consider what they could personally do to improve the lives of people with OI. The founders of Care4BrittleBones, parents of a daughter with OI and members of the VOI, volunteered to support research. Quickly it turned out that the VOI's statutory objectives did not include research. The VOI therefore suggested to set up a separate organisation and work together! And this is exactly what happened: The VOI and Foundation Care4BrittleBones serve complementary goals and support each other in various ways. While the VOI focuses on "making the best of today" by working with the healthcare providers in the Netherlands and organising meetings between people with OI, Care4BrittleBones aims to contribute to a better quality of life "tomorrow" by enabling research and working around the globe.

Care4BrittleBones is present at all formal meetings of the VOI and is regularly invited to address VOI members during plenary meetings with a presentation about the activities of the foundation. Likewise, the VOI is kept up to date about the foundation and they are invited to attend the monthly meetings of Care4BrittleBones whenever they want. The connection is positive and strong and we will continue to support each other in topics of shared interest.

In 2019 Foundation Care4BrittleBones actively participated and presented at both VOI – Meetings and worked together successfully on several projects (see for example the psychosocial project).

ZOI – Belgium OI association – Meeting 10 March 2019

One of the most active and warm OI communities we know is the Belgium – Flemish OI association. When they invited Dagmar Mekking to join their annual meeting in March, this was a good opportunity to learn more about them and to share what the Care4BrittleBones foundation was planning for the year. This resulted in even stronger collaboration. For example the ZOI was very active in project Key4OI and the Conference QualityofLife4OI was attended by a large delegation from Belgium.

German DOIG – Meeting 21 – 23 June 2019

In 2019 Dagmar Mekking was invited to attend the annual meeting of German OI Association (DOIG). The meeting was a great opportunity to re-connect with the strong organization that has been very open and supportive towards Care4BrittleBones from the start. The meetings are a great example of family fun and friendship mixed with excellent workshops and presentations.





OIFE / meeting and Conference See, Hear Smile in Riga / Latvia – 13 – 16 June 2019

For many years Foundation Care4BrittleBones has been a supporting member of the **OIFE** (OI Federation of Europe). The OIFE is an umbrella association for organizations dealing with OI in Europe and beyond. They were established in 1993 and have 30 member organisations. Foundation Care4BrittleBones and the OIFE work together on specific projects, such as the conference QualityofLife4OI (the OIFE was part of the Steering Committee). Also the foundation frequently connects to president Ingunn Westerheim as a sounding board and advisor for strategic initiatives of the Foundation.

In June 2019, Dagmar Mekking attended the annual meeting of the OIFE, which took place in Riga, Latvia. The annual meetings of OIFE are a perfect way to stay connected with other member organisations. The meeting in Riga was combined with a very interesting symposium on hearing, seeing and smiling covering all kind of OI specific challenges around teeth, jaw, hearing and vision, which are critical to the quality of life for people with OI.

OIF Osteogenesis Imperfecta Foundation

The Osteogenesis Imperfecta Foundation is the most important OI association in the USA and is strongly linked to the Canadian COIS. With a very successful track record of almost 50 years of dedicated work of volunteers and strong support from their healthcare providers and researchers, they are a thriving and empowering network that we learn a lot from. Foundation Care4BrittleBones and the OIF work together on specific projects, such as the conference QualityofLife4OI (the OIF was part of the Steering Committee and sponsored many speakers with US/Canadian background). Also the foundation frequently connects to CEO Tracy Hart as a sounding board and advisor for strategic initiatives of the Foundation.





| SCIENTIFIC NETWORKS

Collaboration in the medical world is paramount to achieving progress in OI research and ultimately a better quality of life for people with OI. In 2019, Care4BrittleBones engaged with the following scientific professional contacts:

1. **Advisory boards / Annual Grant Scheme:** Due to the Conference QualityofLife4OI we paused our grant scheme for 2019. We will pick it up in 2020 again.
2. **ERN-BOND:** The European Reference Networks, coordinated by the European Union, advocate for increased collaboration across the 27 member states to support and address rare diseases. In 2019, they continued to develop their network and mature their working processes. Twenty-four networks have been established, each of them focusing on a cluster of rare diseases. OI is part of the ERN-BOND, which covers more than 400 rare bone diseases. OI is one of the most important rare bone diseases, which is why it has been selected as one of the key focus areas of ERN-BOND.
The ERN-BOND kindly agreed to participate in the steering committee of Key4OI and Conference QualityofLife4OI, represented by their coordinator Luca Sangiorgi. More information can be found on <http://ernbond.eu/>.
3. **BBDC:** The BrittleBonesDiseases Consortium is one of the most important networks in the US/Canadian region for research into OI. They were kind enough to brand-sponsor the Conference QualityofLife4OI, providing also the chairman of the conference, Frank Rauch. Also we have partnered closely with OIF (described under 'OI-community'). More information can be found on <https://www.rarediseasesnetwork.org/cms/BBD>
4. **Dutch OI Group:** We are connected to the Dutch OI Group, a group of 15 medical specialists who support OI in the Netherlands. The experts of this group are associated with the Wilhelmina Children's Hospital Utrecht (specialising in children with OI), Isala Hospital Zwolle (specialising in adults with IO), the University Medical Center of Amsterdam and several other hospitals. Together they ensure that they regularly share knowledge with each other and relevant stakeholders, including Care4BrittleBones.
5. **Meetings and Conferences:** In 2019 we participated in the following international meetings, which included networking with researchers supporting OI:
 - a. **ZOI (Belgium OI community) – Scientific presentations**, Belgium – March 2019
 - b. **ICHOM Conference “measuring outcomes”** NL – May 2019
 - c. **DOIG (Germany OI community) – Scientific workshop and presentations**, Germany – June 2019
 - d. **OIFE meeting and „See/Hear/Smile“-conference**, Latvia – June 2019
 - e. **Conference QualityofLife4OI**, Amsterdam/NL - 22-25 November 2019.
This event is described in detail under the paragraph of “research” and was our most impactful scientific activity this year, alongside the Key4OI project and the “psychosocial aspects of OI”-project.
 - f. **The Pegasus Networking meeting** – NL – December 2019.
6. Over the course of the last 8 years, we have established a worldwide **database of about 900 OI researchers and health care providers across all disciplines** supporting people with OI. This includes, for example, orthopaedics, endocrinologists, geneticists, physiotherapists, and dental, hearing and breathing experts. We are in contact with **all leading researchers** in the world. We know them and they know us.
7. **The global Physical Rehabilitation Network:** In the context of a research project lead by Dr. O. Semler in 2017, Foundation Care4BrittleBones has established a global network of experts in physical rehabilitation for OI. The purpose of this network is to meet on a regular basis, exchange knowledge and to deepen the



expertise we collectively have about this area, which is critical for quality of life of people with OI. The Physical rehabilitation network has come together twice in 2019 for topical webinars, which have been recorded and are available on the Care4BrittleBones YouTube channel:

- a. February 2019: Webinar “Tips and Tricks for daily living” – lead by Kathleen Montpetit
- b. April 2019: Webinar “Rehabilitation Strategies in OI”- lead by Mariapia de Bari”

The Network now operates under the leadership of Kathleen Montpetit from Canada.





| COMMUNICATION VIA MULTIPLE MEANS

We are using a multi channel approach for engaging our communities. The main goals are to create and continue solid foundations and create impact, engagement & awareness. We strive for integration of all platforms and support research, fundraising and collaboration where possible. Below is an overview of our various channels.

Website C4BB <http://www.care4brittlebones.org/en/>

Our website contains information about OI, provides an overview of the projects we are conducting and provides information on the foundation and its processes. On top of this "internal" information, it also supports an information platform catering for the needs of researchers, clinicians, healthcare professionals, OI communities and industry supporting OI. There is a possibility to make a donation to support research and show various opportunities to help in other (non-monetary) ways. Our website is updated regularly with new events and is used as an archive. In 2018 we have started our Information Platform, that we have extended in 2019 and continue to grow. In 2019 we have made our website even more accessible and user friendly.

Care4BrittleBones aims to improve the quality of life of people with Brittle Bones Disease



Osteogenesis Imperfecta (OI) ▾

Research ▾

Care4BrittleBones ▾

Information Platform

Donate

Contact



**I am Chedda
and I have OI**

Newsletter

During 2019 we have sent out 19 newsletters about a variety of topics. One of the main events in 2019 was the conference QualityofLife4OI, and therefore a significant number of the newsletters have been dedicated to this. After proven success and positive feedback from the community, we continue to send out one newsletter per month (compared to one newsletters per quarter in prior years).



Instagram & Facebook

We have started with Instagram in 2019, and it has proven to be successful already. We experience engagement and positive feedback with our community. By sharing our Instagram posts directly to Facebook, we reach an even bigger audience. Facebook remains a separate channel for less visual messages and communication within (closed) groups such as “Ouders met OI”/“Parents with OI”.

LinkedIn

The variety in channels gives us the opportunity to target different audiences. We use LinkedIn for professional messages in the widest sense.

YouTube & Webinars

We use YouTube as an archive for our webinars and other video material. During the conference, our ambassador Danielle de Bakker has created multiple vloggs about her experience of the conference. The vlogs are also directly accessible via our website.

Soundcloud

During the conference our ambassador Michael Roele created several great podcasts. They are also directly accessible via our website.

Kentaa / Research4OI.org

Our fundraising platform is a very easy and accessible tool for individuals or team to set up their fundraising event. It also offers information about the foundation and OI.



[Home](#) | [Tips](#) | [Project: Key4OI](#) | [Care4BrittleBones](#)

ACTION PLATFORM RESEARCH4OI



Plans 2020

In 2020 we will grow our online database by actively motivating people to sign up for our newsletter. This will be done by an online integration with our website. In addition, we will include Twitter & Spotify to our channels.

The Team

The communications team consists of volunteers and a number of them have professional communications experience. In 2019 Aleksandra Wrobel stepped down as a board-member and Celine Disch followed in her footsteps as the board member responsible for Communications. Together with the communications team she makes the foundation visible for different audiences and enables information sharing through multiple channels.



FINANCIAL REPORT

| SUMMARY OF FINANCIAL RESULTS INCLUDING EXPLANATION

Since the creation of our Foundation until 2016, Care4BrittleBones managed to generate around EURO 90,000 per annum based on a volunteer model. In 2017 the Foundation changed its operational model and introduced a full-time resource. On average over the past three years, this has allowed us to increase fundraising and funding for research by a factor of four.

2019 was a special year for our Foundation. We planned to keep our normal operations running while organizing the international Conference QualityofLife4OI. Just over half of the income we generated was linked to the conference. Combined with tightly managed spend, the financial outcome of the conference was break-even. On top of this we received another large subsidy of Zorginstituut Nederland and delivered on the expected income. On the other hand, we managed to keep focus on delivering impactful research. Most of our resources were geared on research support or research projects. While our income is slightly higher than budgeted, our spend on purpose was significantly higher than budgeted, driven by a much higher spend on research. Fundraising and Management expenditure has decreased resulting in 4% and 2% respectively of total spend in 2019, compared to 12% and 7% in 2018. Our overhead costs are consistently and considerably lower than the recommended threshold of 25% for charities in the Netherlands.

Income

We delivered on planned income with overall income of ca EUR 441,000 against a 2019 budget of EUR 435,000 (+2 %). The income from individuals and companies in 2019 has been ca EUR 208,800. The key source of income in Income from Grant from Governments was the large subsidy of Zorginstituut Nederland.

On the other hand, our Income from Other Non-Profit Organisations is heavily influenced by a correction of a 2018 commitment. This relates to the “Guideline for good clinical care for OI” project led by ERN-BOND. ERN-BOND received new information from the EU in 2019 which means that the development of the guideline cannot be funded by a charitable foundation but only by EU itself. In this light the Foundation could no longer fulfill its intent to provide funding.

A part of this funding came from one of our sponsors and in 2019 we reversed the originally committed external funding (-/- EUR 80.000). Despite the project being cancelled, the sponsor committed a part of the original amount to us in 2020 (EUR 32.000), recognizing the progress that had been achieved towards the goals.

Total income continues to be heavily dependent on a few significant grants, as long-term donor support is relatively modest. While the feedback is generally positive about the quality of the proposals. Most institutions have a clear preference in prioritizing larger diseases (e.g. cancer) over rare diseases. Despite this, we will continue our efforts to find suitable long-term donors in 2020.



A significant part of our income continues to be generated via fundraising events: the “Dam tot Dam run”, the Spijkerbroekengala 2019 (although this will be booked in 2020), the School run in Germany, and many great individual contributions.

Spend on Purpose

The Spend on Research projects and purpose-related activities of EUR 522,800 is significantly above the 2019 budget of EUR 409,000. The main drivers for this are higher than budgeted spend on Key4OI related activities, for which the first phase was successfully completed. Also, we saw higher spend on the project on Psychosocial care for OI, which was also successfully completed in 2019. Furthermore, there was an impact of the changed timing of the project Towards a therapy for OI - Next Steps. The funds needed for these projects were available from our earmarked reserves.

This higher spend was partly compensated by lower than budgeted costs for our conference. With a strong focus on content, we managed to reduce spend on overheads and non-core programme activities to make sure the overall cost and income for the conference remained in balance after lower than anticipated income from ticket sales.

Fundraising

The cost of fundraising of EUR 20,620 has been significantly lower than the 2019 budget of EUR 31,500 (- 35%) mainly due to a decrease in the number of and size of internally managed fundraising events. Also, the hours spent by the director on fundraising has been lower than anticipated (-39%) and higher on purpose related activities instead.

Management & Admin

The 2019 budget of EUR 24,750 has been underspent (-51%), mainly due to less hours spent by the director on Management & Admin, lower travel costs, and was partly offset by the remuneration of a volunteer with OI.

Financial Position

The Financial Position of the Foundation has remained strong during 2019. Donations are still largely on an ad-hoc basis, we therefore commit to the funding of new research projects and activities only after receiving cash from donations or firm commitments to receive cash in the near future. Due to the focus on the conference, we did not launch a new proposal round in 2019. Combined with the cancellation of the project on Consensus for good clinical care, this has led to a shift of funds on our balance sheet from Earmarked Reserves and Earmarked Funds to Other Reserves.

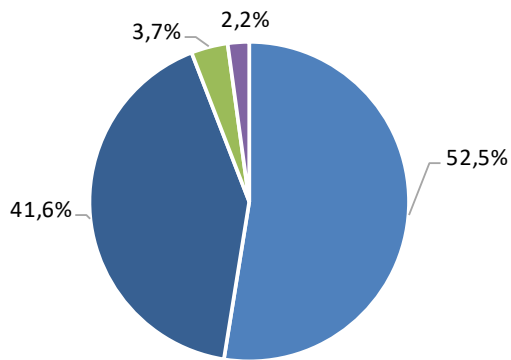
Our Contingent Reserve is more than sufficient to cover the committed cost of the organization in 2020. In addition, we expect to need ca EUR 50.000 from our Other Reserves to cover costs for projects and activities that we started before 2020. However, these commitments are not firm enough to allocate to Earmarked Reserves and Earmarked Funds.



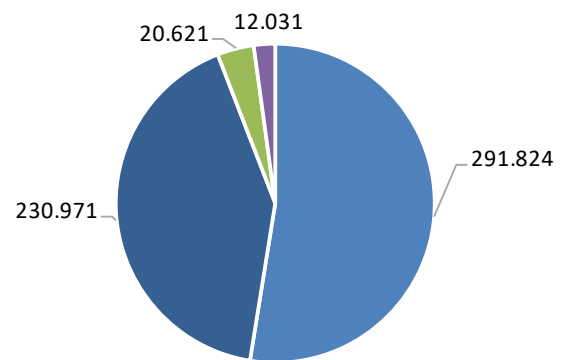
KEY NUMBERS

How did we spend your contribution?

Spent in 2019: €555.447



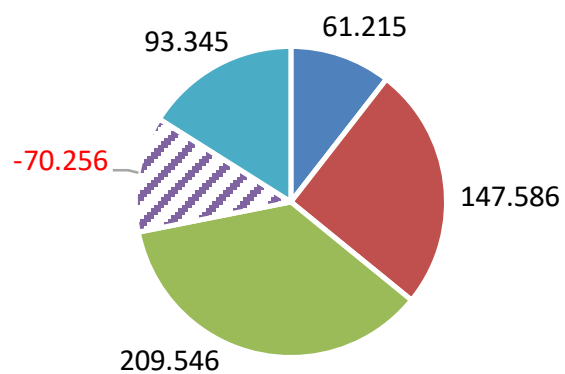
- Research (purpose)
- Info sharing & Collaboration (purpose)
- Fundraising
- Management & Administration



- Research (purpose)
- Info sharing & Collaboration (purpose)
- Fundraising
- Management & Administration

What are the sources of income to fund research for OI?

Income in 2019: €441.436



- Donations from Individuals
- Grants from Government
- Products and/or Services
- Donations from Companies
- Donations from Non-Profit Orgs



| BUDGET 2020

The Care4BrittleBones budget for the following year is approved annually by the Board. Normally, this is based on a balanced operation, i.e. with a change of the Contingent Reserve of zero. There could be balances on designated reserves and funds. These are actually balance sheet transactions that arise from timing differences in income and expenses. Often the Foundation first receives the benefits and then only spends the funds and / or incurs them. Given the significant Other Reserves on our balance sheet, the Foundation has room to prudently spend more than the income it generates. This means that the 2020 budget approved by the Board aims for a negative result of EUR 41.600.

Income

2020 will be a year in which the Foundation aims to 'return to normal' after a year that was centered around the conference. Given the uncertain socio-economic outlook, we looked at the average income over the past 3 years and took this as the baseline for the 2020 budget. The focus will be on generating income from non-profit organisations and individuals with the aim to fund much needed research and an eye out for a potential follow-up event to our 2019 conference.

Expenses

Foundation Goals

Funding of (research) projects takes place based on annual proposal rounds. 2019 focused on enabling the delivery of projects from earlier proposal rounds and on the conference. In 2020 we plan to kick off a new proposal round with a focus on fundamental research, including decisions on what projects will be awarded funding.

We have budgeted to spend a significant amount of our non-earmarked reserves and yet to be generated income on this new research. The financial implication of this could be either costs that hit our P&L in 2020 or commitments in our Balance Sheet to fund research that would impact our balance sheet.

With this, the percentage of spend on Foundation Goals is expected to be 88%. This is a slight decrease from 2019 when we spent an exceptional 94% of our expenses on our goals thanks to the conference.

Fundraising

The costs of fundraising are expected to increase from 4% in 2019 to 7%. This is caused by the expectation that the competition for funds will increase, given fundraising events are generally difficult in 2020 given the Corona/COVID-19 precautions. This means we expect to need to invest more time and effort in generic fundraising activities. The amount budgeted is in line with previous budgets and pre-2019 actuals. Logically we will only proceed with these plans if the fundraising benefits are expected to firmly offset the associated costs.

Management & Admin

The cost of Management & Admin is expected to increase from 2% in 2019 to 4% in 2020. While underlying costs are expected to stay in line with previous years, the amount of time the director spent in 2019 on the organization of the conference and specific research projects was extraordinarily high and is expected to come back down to pre-conference levels in 2020.



Budget 2020 (continued)

Income & Expenses	Budget 2020		Actuals 2019	
	EURO	EURO	EURO	EURO
Income				
Income from Individuals		70,000		61,215
Income from Companies		120,000		147,586
Income from Lottery Organizations		-		0
Income from Grants from Government		10,000		209,546
Income from Associated (international) Organizations		-		0
Income from other Non-Profit Organizations		200,000		-70,256
Total Acquired Income		400,000		348,091
Income as compensation for the delivery of:				
- Products and/or Services		-		93,345
- Other income		-		0
Total Income		400,000		441,436
Expenses				
Spent on Goals				
- Research	377,015		291,824	
- Information sharing & Collaboration	12,500		230,971	
- Other	-		0	
		389,515		522,795
Fundraising costs		32,500		20,621
Management & Administration costs		19,570		12,031
Total Expenses		441,585		555,447
Balance before Financial Income & Expenses		-41,585		-114,011
Balance Financial Income & Expenses		0		3
Balance Income & Expenses		-41,585		-114,008



FINANCIAL STATEMENTS 2019

Income Statement

Income & Expenses	Notes	Actual 2019		Budget 2019		Actual 2018	
		EURO	EURO	EURO	EURO	EURO	EURO
Income							
Income from Individuals			61,215		81,500		97,908
Income from Companies			147,586		55,000		23,539
Income from Grants from Government			209,546		10,000		-
Income from other Non-Profit Organizations			-70,256		90,000		165,930
Total Acquired Income			348,091		236,500		287,377
Income as compensation for the delivery of:							
- Products and/or Services			93,345		198,000		-
Total Income			441,436		434,500		287,377
					101.60%		
Expenses							
Spent on Goals							
- Research		291,824		65,000		159,993	
- Information sharing & Collaboration		230,971		344,000		38,930	
- Other		0		0		-	
			522,795		409,000		198,923
Fundraising costs			20,621		31,500	65%	30,390
Management & Administration costs			12,031		24,750	49%	16,677
Total Expenses			555,447		465,250		245,990
Balance before Financial Income & Expenses			-114,011		0		41,387
Balance Financial Income & Expenses			3		0		99
Balance Income & Expenses			-114,008		0		41,486
Assignment Balance Income & Expenses							
Addition to / Withdrawal from:							
- Other Reserves			-24,419		0		-52,292
- Earmarked Funds			-89,589		0		93,778
			-114,008		0		41,486

Balance Sheet

	Notes	31-Dec-19		31-Dec-18		31-Dec-17	
		EURO	EURO	EURO	EURO	EURO	EURO
Assets							
Receivables & Accrued Assets	1	253,574		200,896		182,557	
Cash & Cash Equivalents	2	259,930		394,254		436,502	
			513,504		595,250		619,059
			513,504		595,250		619,059
Liabilities							
Reserves & Funds							
- Reserves							
- Earmarked Reserves	3	20,415		118,200		0	
- Other Reserves	3	121,533		48,166		100,458	
			141,948		166,366		100,458
- Funds							
- Earmarked Funds	4		101,459		191,048		215,470
			243,406		357,414		315,928
Long Term Debt	5		100,358		75,000		92,000
Short Term Debt	6		169,740		162,836		211,131
			513,504		595,250		619,059



Cashflow statement

	2019	2018
<i>Cash collected from:</i>		
Donations	196,162	103,788
Income from Grants from Government	96,707	46,984
Income from other Non-Profit Organizations	2,544	117,004
Products & Services	93,345	1,050
Interest received	103	211
Total cash collected	388,861	269,037
<i>Cash paid for:</i>		
Research projects	262,436	264,299
Key 4OI Conference	235,276	-
Sponsoring events	-	-
Governance costs	4,771	16,677
Fundraising activities	20,702	30,309
Total cash paid	523,185	311,285
Net cash from operations	(134,324)	(42,248)
<i>Cash Balance:</i>	2019	2018
Opening balance ABN AMRO & SNS Bank	394,254	436,502
Closing balance ABN AMRO & SNS Bank	259,930	394,254
Net cash from operations	(134,324)	(42,248)





| NOTES TO THE ACCOUNTS

General

The consolidated financial statements for 2019 have been prepared in accordance with Part 9, Book 2 of the Netherlands Civil Code and in particular the regulations of the Guideline 650 Fundraising Organizations (RJ650). The purpose of this annual account is to provide insight into income and expenditure, and in the financial position of Care4BrittleBones.

Accounting policies for valuation and determination of results

General

Unless stated otherwise, the assets and liabilities are valued at the acquisition price, the manufacturing price or the current value. If no specific valuation principle is stated, valuation takes place at the acquisition price.

The accounting policies used for valuation and determination of results have remained unchanged compared to the previous year.

In order to be able to apply the principles and rules for the preparation of the annual accounts, it is necessary for the management of Care4BrittleBones to form an opinion on various matters, and to make the directives that can be essential for the amounts included in the financial statements. If it is necessary to provide the insight required in article 2: 362 paragraph 1 of the Dutch Civil Code, the nature of these judgments and assessments, including the associated assumptions, has been included in the notes to the relevant financial statements.

Cash flow statement

The cash flow statement has been prepared according to the direct method. The cash in the cash flow statement consists of cash and cash equivalents.

Receivables and Accrued Assets

Debtors and other receivables are initially valued at fair value. Subsequently, these receivables are valued at the amortized cost price subject to a deferral of provisions deemed necessary.

Cash & Cash Equivalents

Cash and cash equivalents consist of current account and saving account bank balances. Cash and cash equivalents are valued at nominal value.

Project obligations

Contractually committed contributions to third parties are recognized as project obligations, split into long-term and short-term obligations. These are defined obligations, which are therefore recognized as expenses, but not yet implemented or for which no reports have yet been received.



Statement of income and expenses

Accounting principles for the determination of the result.

Income is recognized for the amounts received or promised without deducting the costs incurred by the own organization. Income is in principle recognized in the year in which it is unconditionally committed to or, when the former is not possible, when it is ultimately received.

Charges

Projects are often carried out together with research institutions. Defined contributions in the context of research projects are charged to the year in which the contribution was unconditionally contractually agreed. Other expenses are allocated to the year to which they relate.

Cost allocation

The recommendation 'Costs of allocation of Management and Administration' of Goede Doelen Nederland is followed.

By making use of projects in the administration, which are categorized according to the objectives (Research and Information & Collaboration), costs are recorded as much as possible directly.

The remuneration costs of the director are allocated to the goals, fundraising and management and administration on the basis of time writing.

Reserves and funds

The Care4BrittleBones's assets must be subdivided into reserves and funds on the basis of the Reporting on Fundraising Institutions. The Reserves include the contingent reserve and part of the reserves that are further earmarked by the association's board for special spending purposes (so-called "Earmarked Reserves"). Funds include those funds that have been given a specific destination by a third party, called "Earmarked Funds", as a result of which Care4BrittleBones can only spend these funds on this specific project.

The contingent reserve, reported under "Other Reserves", is intended as a buffer for disappointing income or unexpected expenses. With this reserve, Care4BrittleBones can continue its activities in financially difficult times.

As a CBF (Centraal Bureau Fondsenwerving) accredited organisation, Care4BrittleBones is bound by the rules for holding or building up reserves by fundraising institutions, as well as the instructions for how the asset management policy should be accounted for in the annual report. In accordance with the Financial Management Guidelines for Charities of Goede Doelen Nederland, the Association Board of Care4BrittleBones decided to set the standard for the reserve for Care4BrittleBones as a whole at a maximum of 1 times the annual costs of the organization. Based on the 2020 budget, a contingent reserve of a maximum of EUR 115,070 will follow as of 31 December 2019. The realized balance Other Reserves of ca EUR 121,500 is slightly higher than the threshold set by the Association Board. This is caused by the fact that we did not have a research proposal round in 2019, due to the focus on delivering ongoing research and the conference. And the cancellation of the project on Consensus for good clinical care. This meant we released ca EUR 58,000 from Earmarked Reserves and Earmarked Funds to Other Reserves.



As mentioned in the section on Financial Position, in 2020 ca EUR 50,000 from our Other Reserves will be used for ongoing activities (projects and activities that we started before 2020). The board has agreed to use all surplus Other Reserves to fund proposals from our 2020 proposal round, taking into account any additions from newly generated income. Thus, ensuring we maximise our spend on purpose and bring the reserves back in line with our own standard.

Short-term debts have a duration of less than 1 year. The project obligations and commitments to (research) organizations to the financing of projects have a remaining duration of less than 1 year

| NOTES TO THE BALANCE SHEET

Note 1

	2019	2018
Receivables & Accrued Assets	EURO	EURO
Receivables from Individuals	1,280	1,383
Receivables from Companies	30,302	17,560
Grants from Government	185,422	72,583
Interest		100
Receivables from Non-Profit Organizations	36,570	109,370
Total	253,574	200,996

Note 2

	2019	2018
Cash & Cash Equivalents	EURO	EURO
ABN AMRO Current Account	16.079	2.505
ABN AMRO Savings Account	143.949	291.946
SNS Bank Savings Account	99.903	99.803
	259.930	394.254

Note 3

	2019	2018
Reserves	EURO	EURO
balance 1/1	166,366	100,458
Additions	191,048	118,200
Withdrawals	-215,467	-52,292
Balance 31/12	141,947	166,366

The Additions relate to the closing of obligations for closed and cancelled projects. Withdrawals concern the balance of Income & Expenses for 2019 after movement in the Earmarked Funds.



Note 4

	2019	2018
Earmarked Funds	EURO	EURO
balance 1/1	191,048	215,470
Additions	101,459	80,000
Withdrawals	-191,048	-104,422
Balance 31/12	101,459	191,048

The Additions are related earmarked grants for the Key4OI Kompas and the pilot Blockchain projects. The withdrawals concern the movement in commitment (the exceptional adjustment relating to the project on Consensus for good clinical care) and 2019 spent of the projects with earmarked funds.

Note 5

Note 5		
	2019	2018
Long Term Debt	EURO	EURO
Project Commitments	100.358	75.000

The project commitments concern commitments to research organizations for the funding of projects with a remaining duration of more than 1 year.

Note 6

	2019	2018
Short Term Debt	EURO	EURO
Project Commitments	142.416	151.895
Creditors	27.324	10.941
Total	169.740	162.836

The project commitments concern commitments to research organizations for the funding of projects with a remaining duration of less than 1 year.



| NOTES TO THE INCOME STATEMENT

Explanation of Spending

	Spent on objectives					
	Research	Information sharing & Collaboration	Fundraising costs	Management & Admin	Total actual 2019	Total Actual 2018
Project contributions	53.308	30.469			83.776	421.200
Outsourced work	208.361	162.379	5.834		376.574	30.400
Remuneration of director	30.155	38.124	14.787	4.054	87.120	87.000
Office and general costs				7.977	7.977	7.400
Total	291.824	230.971	20.621	12.031	555.447	546.000



PROJECT OVERVIEW– SPENT ON GOALS

Year	Project Title	Category	Lead Country	Institution	Lead researcher	Status	Project Committed 1/1/2019	movement in commitment 2019	Total Commitment 31/12/2019	PIY Paid/ reserve d	Outstanding 1/1/2019	PIY Paid/ received	Directors' remuneration 2019	Total actual to date	Outstanding to be paid 31/12/2019
2014	2001	Basic science	Netherlands	VU Medical Center	Pieter van Dijk	completed	31.947	0	31.947	22.469	9.478	9.478	0	31.947	0
2016	No contract	Clinical research	Netherlands	Wilhelmina Kinderziekenhuis	Bas Zuur and Adria van Dijk	Ongoing	10.000	0	10.000	0	10.000	0	0	0	10.000
2016	2009	Clinical research	Ireland	University of Helsinki	Heddi Aapane	completed	20.000	-7.424	12.576	20.000	-7.424	-7.424	0	12.576	0
2016	2010	Clinical research	Denmark	Aarhus University	Dorte Haubæk	completed	16.350	0	16.350	14.350	2.000	2.000	0	16.350	0
2017	2011	Basic science	Netherlands	Isala / UMC Groningen	Pieter van Dijk	completed	156.600	0	156.600	0	156.600	50.000	0	50.000	106.600
2017	2012	Clinical research	Italy	Politecnico di Milano Dipartimento di Elettronica	Antonella Lo Mauro	completed	35.000	0	35.000	15.000	20.000	0	0	15.000	20.000
2017	2013	Basic science	Australia	The Centre for Children's Bone and Musculoskeletal Health Australia	Craig Morris, Aaron Schneider etc	completed	24.000	2.220	26.220	24.000	2.220	2.220	0	26.220	0
2017	2015	Psychology	Netherlands	Care488	various	completed	170.810	32.000	202.810	91.762	111.048	69.855	11.693	202.810	0
2018	2016	Quality of life for children with OI and Research Priorities	UK	Sherfield Children's NHS Foundation	Liane Hill and Nick Bishop	completed	26.596	-16.772	10.326	5.000	5.326	0	0	5.000	5.326
2018	2017	Basic science	Netherlands	Isala	Anton Franken, Gert Jansen, Ralph Sallers	completed	60.000	-30.000	30.000	0	30.000	13.000	0	13.000	17.000
2018		Consensus for good clinical care	Sweden	Marimónska Institutet, C488	Lars Sörensdahl	cancelled	138.200	-138.200	0	0	0	0	0	0	0
2019	2018	Key401 – Focus Groups (adults and children) and other costs	Netherlands	Care488		completed	0	22.721	22.721	0	0	14.568	7.753	22.721	0
2019	2019	Key401 – children outcome measures / UMC U	Netherlands	UMCU		completed	0	45.000	45.000	0	0	45.000	0	45.000	0
2019	2019-20	Key401 Variant Database	UK	University of Leicester	Raymond Dalgleish	allocated	0	3.415	3.415	0	3.415	0	0	0	3.415
2019	2019-21	Key401 kompas	NL	Care488		completed	0	201.546	201.546	0	201.546	8.837	1.530	10.067	191.479
2019	2019-22	Blockchain pilot voor OI	Netherlands			completed	0	15.000	15.000	0	15.000	0	0	15.000	0
Total Projects							689.466	110.006	819.511	192.581	559.209	237.134	33.143	462.858	367.893
							0	298.697	298.697	0	298.697	204.185	35.136	219.321	0
Overall Total							689.466	418.703	1.118.209	192.581	857.906	441.519	68.279	702.179	367.893



NOTES ON THE ROLE AND REMUNERATION OF THE DIRECTOR

Notes on the role and remuneration of Management	
Name	Dagmar Mekking
Position	Director
Line of work	Contract (2 years)
Hours	40/week
Parttime percentage	100
Period	1/1/2019-31/12/2019
Annual remuneration (gross)*	87120
Vacation payment	-
Year-end payment	-
Compensation holidays not utilised	-
Total	87120
Taxable reimbursement	-
Pension payments (Foundation)	-
Pension compensation	-
Other long term remuneration	-
End of Service payment	-
Total	87120

The total remuneration of the Director (self employed) remains within the maximum of EUR 95.183 (1 FTE/12 months) as per regulation for remuneration of directors of Charity Organisations jobgrade D' from Goede Doelen Nederland applicable in 2019.

The allocation of remuneration costs of the director is based on time writing, see 'Explanation of spending' (page 59).

**including 21% VAT (is not recoverable as our foundation is exempt from VAT).*



| ACCOUNTING CONTROL STATEMENT

2019 control statement:

Accounting Control Statement

The Auditing Committee in the person of Ms E.L. Koster and Mr. R. Le Grand have on Sunday 31st May 2020, in the presence of Treasurer Mr. D.J. Stegink and Mrs. I Juzwa-Parus checked the books of the Care4BrittleBones foundation for the 2019 financial year.


The Committee states that:

- The Annuals Accounts give a correct representation of the income and expenses for the year 2019
- The balance sheet as at 31 December 2019 give a true and fair view of the Reserves.

Den Haag, June 2nd 2020

The Auditing Committee

E.L. Koster


R. Le Grand




THANK YOU!

We would like to thank everyone who directly supported Care4BrittleBones in the last year – our generous donors, fundraisers and ambassadors and other volunteers. The achievements of Care4BrittleBones are enabled by this group of people, working relentlessly together to make a difference for people with OI. A wholehearted thank you to the core team for the numerous brainstorming sessions, discussions, challenges and huge effort committed to our cause. The power and passion of this team makes our organization move forward.

We also would like to thank our partners in research, working in hospitals, universities and other health related institutions around the world. We explicitly include in this also our partners in the industry. Your deep expertise and true dedication to improve the lives of people with OI and other rare bone diseases are an inspiration to us. We admire your innovative ideas and your courage to try out new approaches in responsible ways to take away pain and fractures. Your work gives us hope for a better future.

And of course – a big thank you to our wider OI Community network, all people with OI, their families and friends who have supported us in 2019! A very special thank you is for OIFE and OIF, who work shoulder to shoulder with us on various projects. We are extremely proud to be associated with you and thankful for the active role you take together with us to improve quality of life for people with OI!

We stand strong together!





CARE4BRITTLEBONES | FOUNDATION

Care4BrittleBones (statutory name 'Stichting Care4BrittleBones') is located in Wassenaar. It is registered as a foundation (Dutch: 'Stichting') at the Chamber of Commerce of The Hague Nr. 54665256.

Established as a foundation in February 2012, Care4BrittleBones is recognised as a charitable organisation in the Netherlands since 12 June 2012 (RSIN 851392854; www.kennisbankfilantropie.nl/anbi/care4brittlebones).

Based on a ruling of the European Court of Justice, the recognition as charitable institution in one member state is sufficient to establish beneficial tax treatment for donations from donors based in any country of the European Union.



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 YouTube	www.youtube.com/c/care4brittlebonesOrg070
 LinkedIn	www.linkedin.com/company/10639515