# Table of Contents

- President’s Statement: 1
- The Organisation: 3
- The Administration: 6
- OIFE Events: 9
- OIFE Projects: 13
- Representation, Advocacy & Networking: 17
- Communication & PR: 25
- Research & Development: 30
- Peer Work: 32
- Financial Status: 35
- Summary and Thank You: 37
In 2023 we started the same way as in 2022 – with an OIFE Leadership Meeting on Zoom. The purpose of the meeting was to inform our members about future events and activities.

This included patient involvement in clinical trials, access to treatments, OIFE campaigns in 2023 and more. The Pain and OI survey was launched at the meeting, and it was kept open until April 1st.

A lot of time and energy in 2023 went into OIFE’s 5th Topical Meeting “Balancing Life with OI”, which took place in Stockholm in June. The main topic of the international conference was pain in OI and its impact. We raised a lot of funds for the meeting, both from industry grants and an EU grant of €30,000. Together with participant fees, this gave as a total income in 2023 of ca €200,000 euros.

The work paid off, and the event was very successful – with ca 180 participants on site and ca 40 online. The audience was a mix of clinicians, researchers, OI community members, rare bone representatives and industry. 98% of those who answered the evaluation, said the conference met their expectations. The meeting was followed by our annual general assembly.

The first half of 2023 we hosted monthly Drop-In sessions, to create an arena for exchange between OIFE members. But due to a lack of interest and numbers of attendees being low, we decided not to continue with these. More successful events included the OIFE Youth Event in Italy, the Clinical Trial Update Webinar (online) and the OIFE Investigator Meeting (online). We were also represented at several other events, where the biggest one was the orphan drug conference in Barcelona.

More research than ever is going on in OI. Several new therapies are in the pipeline, and we need to educate ourselves and our members on what we can do to secure access to these therapies in the future. Every year we get to know new stakeholders interested in developing potential therapies for OI. We try to connect with them quickly, because patient involvement needs to begin early, to have a real impact. In 2023 we provided input to several central clinical trials in OI, including Cosmic and Orbit (Mereo & Ultragenyx), Poise 1 (Sanofi), Romosozumab (UCB & AMGEN), TOPAZ and BoostB4.
We have also invested a lot of time in patient experience data (PED) and real-world evidence projects like the IMPACT survey, the Saturn project and the care pathways project.

The activity in OIFE is increasing every year. The only challenge is to keep up with everything that is happening both timewise, but also knowledge wise. Thankfully we have more opportunities to outsource some of our work, due to more funding.

Ingunn Westerheim – OIFE president
THE ORGANISATION

OUR VISION, MISSION & GOALS

Our mission is connecting and empowering organisations, professionals and individuals to improve the lives of people with OI.

International networking is still the most important activity for OIFE. We keep ourselves informed by attending events, talking to all kinds of stakeholders, and bringing the right people together, when needed.

Our vision: Children and adults with OI living active and independent lives - with access to competent healthcare and necessary social support.

In accordance with our strategy plan the OIFE general assembly in June 2023 approved the main aims for OIFE to include:

- increase awareness on OI and its impact.
- advocate for the OI community on a European level.
- empower, educate and inform the OI community.
- grow an international network of interested stakeholders.
- encourage advancements in research.
- promote patient involvement on different levels and areas.
OUR MEMBER ORGANIZATIONS

OIFE had **41 member organizations at the end of 2023.**

This included **20 European national organizations** with full voting rights. These countries are:

- Austria
- Belgium
- Denmark
- Finland
- France
- Georgia
- Germany
- Italy
- Latvia
- The Netherlands
- Norway
- Poland
- Portugal
- Romania
- Russia
- Spain
- Sweden
- Switzerland
- UK & The Republic of Ireland
- Ukraine

We had **16 associate members**, who are national organizations outside Europe without voting rights:

- Australia
- Brazil
- Canada
- Chile
- China
- China Zero One
- Ecuador
- Ghana
- India
- Kazakhstan
- Mexico
- Nigeria
- Panama
- Peru
- South Africa
- USA
We welcomed two new associate member organizations in 2023:
- Brazil (Associação Nacional de Osteogenezese Imperfeita ANOI)
- CLAN (Caring & Living as Neighbours) seated in Australia

In addition to the non-European associate members, we had five supporting member organizations consisting of research foundations (AHUCE Foundation, Care4BrittleBones and the Belgian AFBOI) as well as one organization providing aid & support for individuals (Padrinos).

CLAN became OIFE’s first NGO member.
THE ADMINISTRATION

THE GENERAL ASSEMBLY

The highest authority of the OIFE is the General Assembly, which normally comes together once a year.

It consists of one delegate from each member organization. Only European members have voting rights, but all official OIFE representatives receive invitations and the same information.

THE BOARD

The General Assembly appoint members of the Board, who runs the day-to-day business of OIFE. Our Board member Renata Hoes resigned after a short time period in January 2023.

The rest of 2023, the Board consisted of the following people:

**PRESIDENT:**
INGUNN WESTERHEIM
NORWAY
RE-ELECTED IN 2023 UNTIL 2027

**VICE-PRESIDENT:**
BRUNO VAN DIJK
BELGIUM
ELECTED UNTIL 2024

**TREASURER:**
COREEN KELDAY
UK
ELECTED UNTIL 2025

**COMMUNICATION MANAGER:**
ANNA ROSSI
ITALY
ELECTED UNTIL 2024

**BOARD MEMBER:**
MALENE SILLAS
DENMARK
ELECTED UNTIL 2026

**BOARD MEMBER:**
STEPHANIE CLAEYS
BELGIUM
ELECTED UNTIL 2026

**BOARD MEMBER:**
JACOB WITTLORFF
DENMARK
ELECTED IN 2023 UNTIL 2027
The Board had 11 online meetings in 2023 and two face to face meetings from April 28–30 in Stockholm, Sweden and from November 25–26 in Mechelen, Belgium.

THE VOLUNTEERS

The Board is supported by the following formally appointed volunteers:

**SECRETARY:**
Stefanie Wagner - Germany

**CHAIR OF OIFE MAB:**
Oliver Semler - Germany

**WEBMASTER:**
Kevin Vanantwerpen - Belgium

**ANDRE WITTWER:**
Norway

**SOCIAL NETWORK COORDINATOR:**
Ute Wallentin - Germany

**Maria Barbero:**
Spain

**OIFE YOUTH COORDINATOR:**
Stephanie Claeys - Belgium

**Simey Truong:**
Germany

Matilde Medum Nielsen - Denmark/Australia

**ERN BOND EPAGS:**
Rebecca Tvedt Skarberg - Norway

Claudia Finis - Germany
HONORARY MEMBERS

OIFE’s Board is grateful to the ongoing support and advice provided by honorary members Ute Wallentin and Taco van Welzenis. In addition to the formal volunteers, OIFE is supported by other volunteers on project & ad hoc basis.

OIFE MEDICAL ADVISORY BOARD

There were no changes of members in the OIFE Medical Advisory Board (MAB) in 2023. The Chair of the MAB continues to be Dr. Oliver Semler. The MAB advice the OIFE on topics such as increased number of requests for support from research projects and consortiums preparing grant applications. Members of OIFE MAB were also actively involved in the planning and implementation of OIFE Clinical Trial Update and the OIFE Investigator Meeting. We also representation at various Medical Events from members of our MAB throughout the year.

PAID SUPPORT

We secured funds to assist with buying professionals services from several different professional providers in 2023. We contracted Janet Crompton to provide support with event planning and Gabriela Beug for writing up reports in relation to the Events and in particular the Pain Project.

We continued to pay for the ongoing services from SBB in Belgium in relation to Accounts and Legal advice, and also the continued secretarial support from Stefanie Wagner.
OIFE EVENTS

JAN

OIFE LEADERSHIP MEETING

On January 23rd the member organizations of OIFE were invited to the OIFE Leadership Meeting 2023. The purpose of the meeting was to inform the organizations about events and activities going on in the OIFE. Ca 35 participants took part from 18 different OIFE member organizations.

The agenda included the following topics:

- News from OIFE incl. clinical trials
- Launch of OIF & OIFE pain survey
- EuRR–Bone registry – status and need for assistance from OIFE members
- How are medicines approved in your country and why you should know?
- OIFE campaigns 2023
- How and why collaborate with healthcare providers? Example from ZOI

OIFE TOPICAL MEETING

“BALANCING LIFE WITH OI”

The 2023 OIFE Topical Meeting took place from June 9–10 in Scandic Continental hotel in Stockholm, Sweden. It was the first conference of its kind to cover topics such the impact of pain on fatigue, sleep, mobility, family, relationships and more.

We had participants from 20 different countries, which included 180 participants on site and ca 40 participants on streaming. Attendees included healthcare professionals, researchers, pain specialists, people from the OI-community and rare bone community and industry. The meeting was organized by OIFE, with some support from the Swedish OI-organization SFOI. The chair of the meeting was Dr. Lena Lande Wekre from Norway.
In addition to 2 volunteers from OIFE, members of the programme committee included OI professionals from Norway, Sweden, The Netherlands, Estonia and Croatia. The main task of the programme committee was to assist with programme planning and a grant application for the European Joint Programme of Rare Diseases (EJPRD). The grant application was successful and brought us €30,000 in project funding for the event.

The meeting offered an opportunity to present and hear about the latest research, treatment methods and news related to pain and OI. The programme included assessment, measurement and management of physical and psychological challenges due to pain in OI and other rare bone diseases.

The conference was a huge success with 98% of those who answered the evaluation (88 people), saying the conference met their expectations.

“A well-organized event, range and knowledge of speakers was impressive, a fantastic opportunity to network and bring together the rare bone disease community.
A stimulating and informative event, thank you ever so much OIFE.”

“I just want to say THANK YOU from the bottom of my heart for doing this meeting. I don’t know any of you behind this project, but I can honestly tell you that since I booked my ticket back in February I have felt less alone in this difficult battle.”

WHAT IS YOUR OVERALL ASSESSMENT OF THE EVENT
88 RESPONSES

- Excellent: 52.3%
- Very good: 45.5%
- Good: 2.5%
- Fair: 0.6%
- Poor: 0.6%
OIFE ANNUAL GENERAL ASSEMBLY

Our Annual General Assembly 2023 was held at Scandic Continental in Stockholm, Sweden on the 10th and 11th June 2023. The formal business part took place on Saturday afternoon after the topical meeting. In parallel, the Swedish OI-organization had an educational seminar on OI with contributions from international speakers. AGM Topics included the normal business part and a new list of strategic goals for OIFE. On Saturday evening we had a very nice dinner, where we also made a toast for OIFE’s 30th anniversary. On Sunday we had an open discussion with evaluation of the events and ideas for activities and priorities for the future.

OIFE INTERNATIONAL YOUTH EVENT IN ITALY

From September 7th - 10th 2023, it was once again time for the young people of the European OI community to come together. This time in Lido di Jesolo, in the Veneto region of Italy, one hour away from Venice. The event was organised by the Italian OI organisation Associazione Italiana Osteogenesi Imperfetta (ASITOI) with support from OIFE youth coordinators, and 30 participants from 12 countries made their way to the sunny Italian coast.
OIFE CLINICAL TRIAL UPDATE

October 24 we managed to gather 125 people from a large number of countries on Zoom for our OIFE Webinar about Clinical Trials.

The goal of the webinar was to update the OI-community about the following clinical trials and projects:

- The Cosmic and the Orbit trials (setrusumab)
- The BoostB4 trial (stem cells)
- The Topaz trial (teriparatide and zolendronate)
- The Poise I study (SAR439459)
- The Saturn project (real world evidence)

OIFE INVESTIGATOR MEETING

On the 17th of November 2023, OIFE hosted the second Virtual OIFE Investigator Meeting for the OI research community. 273 people from 48 countries signed up and more than 160 individuals attended the online event. Attendees were a mix of health professionals, scientists, OI clinicians in addition to a lower number of representatives from industry and patient groups.

The programme committee included researchers from Austria, Spain (two) and Denmark and one patient expert from Norway.

A report from the meeting was written and disseminated to the participants.

The programme included the following topics:

- Research collaboration in Europe: hurdles and opportunities
- What’s new in basic science?
- Methods and tools to evaluate outcomes in clinical trials
- Nosology and classification in OI

WHAT IS YOUR OVERALL ASSESSMENT OF THE EVENT
74 RESPONSES

- Excellent: 58.1%
- Very good: 9.5%
- Good: 32.4%
- Fair: 0%
- Poor: 0%
- Not sure: 0%
OIFE PROJECTS

THE PAIN AND OI PROJECT

THE PAIN & OI RESOURCE GROUP

The Pain & OI project, is a collaboration between the OIFE and the OIF (USA). It includes an international resource group on pain & OI. In 2023 the multidisciplinary group included ca 30 people from 12 different countries, covering both paediatric and adult professionals.

The group include PTs, orthopaedic surgeons, endocrinologists, rheumatologists, anaesthesiologists, pain specialists, psychologists, paediatricians, researchers, nurses and a social worker. In addition, the group also included staff members of OIF, adults with OI and parents of children with OI.

We organized two workshops with the group in 2023, in January and March.

THE TOPICAL MEETING ON PAIN AND ITS IMPACT

Under the title “Balancing life with OI” we organized a topical meeting on the neglected subject of the causes, assessment and management of pain in OI. Many of the participants in the Pain and OI resource group also contributed as speakers at the meeting. See more under OIFE Events.

Most of the talks and presentations from the topical meeting was made available with English subtitles in a dedicated playlist on OIFE’s YouTube: https://youtu.be/OnyNZPRn0dw

The abstract book and reports from the meeting were published on our website. We also published a photo gallery. Mereo Biopharma sponsored the professional photographer, Martin Bodvidsson.

In 2023 we also started the work of writing up an expansive report with key take home messages from the conference. We hired a medical writer to help us. The aim is to use this report when developing a toolbox about pain and OI.
THE PAIN AND OI SURVEY

The pain and OI survey was created by the OIF with input from OIFE and the resource group. The survey was in English language for people with OI who were 18 years old or older. The aim was to learn more about the different types of pain people experience, severity of pain and frequency, and how it impacts people’s lives. We recruited 1088 responses globally. Researchers from Baylor’s College of Medicine has volunteered to write an article with findings from the survey.

OTHER PAIN RELATED PROJECTS

OIFE has also been involved in an industry-initiated project called “Qualitative Research on Patient Experiences of Pain in Osteogenesis Imperfecta. An interview-based study.”

The project wants to create a better understanding of the signs, symptoms, and impacts of OI with a focus on pain, fatigue, and physical functioning. The goal is to develop better tools to measure meaningful change in clinical trials. OIFE (Ute and Ingunn) and OIF (Tracy Hart) has been part of the steering committee and organizations in UK, USA, Australia and Spain were asked to support the project.

The pain and OI project has been successful in sparking an interest in doing more research on the topic of pain and OI and several projects have been initiated during the project.

THE IMPACT SURVEY

The IMPACT survey is an international research project exploring the impact OI has on people’s lives. The project was established in April 2020 and is a joint initiative between the OIFE, the OIF & the company Mereo BioPharma. Five central OI-professionals from Europe and US/Canada are part of a steering committee led by Dr. Frank Rauch. Our goal is to use data from IMPACT to enable better treatment and care, and to support availability of potential future treatments for OI.
Five scientific articles were originally planned from the IMPACT survey. One article was published in 2023: “The patient clinical journey and socioeconomic impact of osteogenesis imperfecta: a systematic scoping review”.

Two more were submitted for publication:

- The IMPACT Survey: Understanding the experience of children, adolescents and adults with osteogenesis imperfecta and their caregivers.
- The IMPACT Survey: The economic impact of osteogenesis imperfecta in adults.

There were several talks and poster presentations about IMPACT at conferences throughout 2023.

The ISPOR conference included three posters from IMPACT:

- Management of osteogenesis imperfecta (OI): self-reported access challenges to consumables and services across the EU5 and Nordics
- A global perspective on self-reported productivity losses associated with osteogenesis imperfecta
- Management of osteogenesis imperfecta (OI): self-reported funding sources for healthcare, consumables and services across the EU5 and Nordics

There were two oral presentations about IMPACT at the OIFE topical meeting and various national conferences in the UK, Italy (2) and France with country specific findings.

Taco van Welzenis and Ingunn attended three meetings in the Steering Committee in 2023. This came in addition to meetings between OIFE, Mereo and Wickenstones about communication on the IMPACT survey.

**STRONGER TOGETHER PROJECT**

OIFE’s member organizations are struggling more and more with various challenges.

These include knowledge of and strategies concerning communication, fundraising, advocacy, recruitment of new members and board members. In several groups there is also a lack of advocacy skills, motivation, experience, resources and volunteers with time and dedication.

There is a real and increasing risk that many of our member organizations are not educated and equipped enough to fill their role as national patient advocacy groups.

The OIFE Board has come up with a project called Stronger Together to tackle these challenges. In the project we will focus on how we can help, support and motivate our member organizations.
“FLYING OI-EXPERTS”

The intention behind “Flying OI Experts” is to bring knowledge to less developed countries.

In 2023 the two OI surgeons Dr. Aldofredo Santana (Venezuela) and Dr. Carlos Pargas (USA) travelled to Lagos, Nigeria to teach and perform surgeries as part of the OIFE Flying OI Expert programme. The project was organized by Osteogenesis Imperfecta Foundation, Nigeria (OIFN) and financially supported by the OIFE and donations from members of NFOI (Norway).

The project was a success, but we realized that we need to develop a better process to manage applications and grants.

A STRONGER BOND BETWEEN US

EUROPEAN RARE BONE FORUM (ERBF)

OIFE kept their position as an informal member of the European Rare Bone Forum in 2023.

There were not a lot of activities in ERBF in 2023. Ingunn was involved as co-author on the project called “A Systematic Literature Review of the Impact and Measurement of Mobility Impairment in Rare Bone Diseases”, supported by the company IPSEN. A poster was presented at the ASBMR conference, and an article was written.

XLH ALLIANCE & OTHER RARE BONE GROUPS

We continued our collaboration with the XLH Alliance in 2023 with two Zoom meetings to share ideas, best practises, and potential collaboration projects. The XLH Alliance also hosted a Board meeting in Stockholm and made valuable contributions at the Stockholm conference.

We invited patient advocacy groups representing the following bone conditions to share their experiences about pain and create awareness about the diagnosis at the Stockholm-conference: XLH, MO/MHE, FOP and HPP.
REPRESENTATION, ADVOCACY & NETWORKING

REPRESENTATION IN FACE TO FACE MEETINGS HOSTED BY OTHERS

THE OIF INVESTIGATOR MEETING IN CHICAGO

Every year the OIF host a Scientific Meeting where researchers and clinicians from North America is invited for a 2-and-a-half-day long seminar focused on OI research.

Lars Folkestad, member of OIFE’s Medical Advisory Board, represented OIFE at the meeting in April.

Read his report here.

PATIENT INVOLVEMENT IN PRECISION MEDICINE

From May 4–5 Ingunn attended a large Norwegian conference about precision medicine (personalized medicine) – where she gave a 30 minute talk about patient involvement – on an individual level, on a policy level and in research and care management. Both researchers, clinicians, patient representatives and policy makers were present.

EU MEETING “A UNION OF EQUALITY”

On June 27 OIFE volunteer Rebecca T. Skarberg attended the EU Meeting “A Union of Equality: Disability Rights and Strategies”, which was a seminar on examples of how to reach equality by using strategies to realize the rights of persons with disabilities.
WORLD ORPHAN DRUGS CONGRESS (WODC) IN BARCELONA

From October 30th to November 2nd, Ingunn, Stephanie and Inger-Margrethe represented OIFE at the World Orphan Drugs Congress (WODC) Europe, where we had free tickets.

WODC is primarily for the industry, but there are also policy makers, NGOs, and other types of stakeholders present. It is a huge conference with more than 2000 participants. Before the main event, we attended a smaller workshop about how better use of data can reduce the impact of rare conditions, where we shared our experiences from the IMPACT survey.

Conference topics included the new European pharmaceutical regulation, advanced therapies, the use of real-world evidence, access to new therapies, and patient involvement in clinical trials, in health technology assessment (HTA) and in development of policies and regulations. The conference was both interesting and thought provoking with excellent opportunities to meet new and old contacts.

OTHER MEETINGS:

In addition representatives from OIFE were present in the following meetings:

- Seminar on challenges and possibilities in advanced therapies, March 13, Oslo, Norway (Ingunn, Rebecca and Inger-Margrethe)
- European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA), October 10, in Amsterdam, (Miguel R. Molina)

We also had OIFE flyers on display at Paediatric Society of the African League Against Rheumatism (PAFLAR) conference in Kenya – April 2023.

REPRESENTATION IN EURORDIS

OIFE volunteer Rebecca Tvedt Skarberg is a board member of EURORDIS. In addition to ca 10 board meetings per year, she is also represented in many other subcommittees.

In the EU Disability Platform, she is a member of a subgroup on Independent Living. And in 2023 EURORDIS established the Mental health and wellbeing partnership network, where Rebecca is in the steering committee.
RARE DISEASE WEEK BRUSSELS

The Rare Disease Week is an advocacy training where the participants learn about policy work, EU advocacy.

It includes meetings with Members of the EU Parliament (MEPs). Rebecca was registered to attend Rare Disease Week in Brussels in February 2023, face to face. However – she had to change her participation to online attendance.

She gave her talk at the EU Parliament event online.

EURORDIS GENERAL ASSEMBLY

Malene Sillas represented OIFE at the EURORDIS AGM (online) on May 17th, where a new board was elected, annual reports approved, and new strategies discussed.

EURORDIS MEMBERSHIP MEETING

The 26th and 27th of May 2023, OIFE attended the EURORDIS Membership Meeting in Stockholm, attended by more than 200 participants.

The workshops were split into different phases of life; Children, Adolescence, Adults and Aging. Rebecca was the moderator for the sessions on adults, and Lars Nesset, represented OIFE as a speaker in a panel discussion about adolescence and rare diseases. Inger-Margrethe Paulsen represented NFOI.

EURORDIS SUMMER SCHOOL

In 2023 physiotherapist Miguel R. Molina graduated from EURORDIS Summer School.

EURORDIS TASK FORCE ON HTA

Inger-Margrethe represented OIFE in the EURORDIS Task Force on Health Technology Assessment (CTA). Through this project, she has acquired important knowledge which our members will benefit from. In 2023 she attended 4 online meetings and one face to face meeting in Paris on October 16th.
EURORDIS COUNCIL OF FEDERATIONS MEETING (CEF) BRUSSELS

On December 13th OIFE was represented with 4 people at the meeting for national alliances (CNA) and federations (CEF) in Brussels. Ingunn attended on site and Bruno, Inger-Margrethe and Rebecca online.

Topics included independent living, the proposed European pharma regulation, access to advanced treatments, cross border healthcare, Rare Barometer surveys and activities in the World Health Organization (WHO).

On December 14 there was a meeting exclusively for federations. We learned about how we can work with industry about the youth project and fundraising of the European Huntington Association. Ingunn attended on site and Inger-Margrethe online.

REPRESENTATION IN ERN BOND & EURR-BONE

ERN BOND

The European Reference Network for Rare Bone Diseases (ERN BOND) brings together 53 health care providers (hospitals) in Europe.

Claudia Finis and Rebecca Tvedt Skarberg represented OIFE as ePags in ERN BOND and they attend a lot of video calls in steering committee and workgroups in 2023. The OIFE was well represented at the spring meeting of ERN BOND in Bologna in May. 40 people attended face to face and 13 online, including OIFE MAB members, Rebecca and Claudia (OIFE) and Inger-Margrethe (NFOI) and Leonardo (ASITOI).

Future plans and priorities in the network were discussed. On May 6th the seminar “Patient priorities in ERN BOND beyond Quality of Life” took place, organized by the patient representatives in ERN BOND.

Suggested priority topics included pregnancy, movement analysis and mobility, transition and pain.
EURR-BONE REGISTRY

On February 13th we were four from OIFE who attended the final meeting of the EURR-Bone registry as a 3 year project. Claudia attended face to face and Taco, Rebecca and Ingunn attended online.

The project team reported delivery on all milestones. OIFE will continue to be actively involved in the groups for the disease specific modules (vertical themes), which are now called study groups.

Four OIFE representatives attended the Study Group Meeting on June 3 (IW, TvW, RTS and CF).

EURR-B, the European Registries for Rare Endocrine and Bone Conditions consists of the EURRECa and EURR-Bone project. On April 3rd the first joint EURRECa / EURR-Bone symposium took place in Leiden, the Netherlands. Claudia attended face to face and others attended online.

In addition to an overview from the perspective of the ERNs, the professionals and the patients, there were also presentations from the disease specific modules – including OI.

OIFE ATTENDANCE AT ONLINE MEETINGS HOSTED BY OTHERS

NORDIC RARE DISEASE SUMMIT

On April 17 OIFE’s president Ingunn was invited to talk at the Nordic Rare Disease Summit (hybrid), which was attended by more than 600 participants – 50% live and 50% virtually. Ingunn attended online and took part as a panellist in the session called Access to innovation.

BILBAO CONFERENCE ON THE FUTURE OF EUROPEAN REFERENCE NETWORKS

On October 11th, EU leaders and key policymakers gathered in Bilbao, Spain, for the Conference on Rare Diseases and the European Reference Networks (ERNs). Discussions addressed some of the main challenges facing the EU policy framework on rare diseases, explored the future of the ERNs. Three representatives from OIFE attended online.
EFPIA PATIENT THINK TANK

November 22nd, we were invited as observers to the hybrid meeting of the Patient Think Tank of the European Federation of Pharmaceutical Industries and Associations (EFPIA). We learned about other federations, hurdles and opportunities for how patient organizations can work with industry, medicine shortages and patient engagement in health technology assessments (HTA) on a European and national basis.

After the meeting, OIFE asked to become a member of the Think Tank.

EUROPEAN HEMOPHILIA CONSORTIUM THINK TANK

In 2023 Ingunn took part in the European Hemophilia Consortium Think Tank on care pathways.

The goal was to map out the challenges and opportunities in care pathways for rare conditions.

Due to colliding meetings and time constraints, Ingunn had to pull out of the consortium half ways.

The report from the first workshop is published and can be read here.
OTHER MEETINGS

Other meetings and conferences where OIFE was represented included:

- 2023 CIOMS-CoRE Webinar: CIOMS Working Group XI Consensus report on Patient involvement in the development, regulation and safe use of medicines, Jan 31 (ISMP)
- EURORDIS Alumni – how to apply to a call for research projects Feb 23 (IW)
- TeleECHO Bone Turnover Markers, March 2 (IW)
- Rare Bone Disease Alliance Scientific Symposium on OI online, March 9-10 (IW and IMSP)
- From Theory to Practice: Implementing the EU Health Technology Assessment Regulation, May 11 (IMSP)
- Open Academy Masterclass – 22 May 2023 – Introduction to QoL and PROMs (IMSP)
- ECTS Webinar Case Studies OI, June 5 (IW)
- Webinar AI and patient voice (Semantic Hub), June 29 (IW)
- “An EU Ecosystem for Rare Diseases: The OD Expert Group proposals for navigating the challenges ahead! – Thursday, 29 June (Claudia Finis and IMSP)
- ECTS eCampus: Interactive Case Discussions: OI in Adults, July 5 (IMSP)
- RARE REV-inar episode 008 – The power of the patient voice in shaping policy and advancing research, July 19 (IMSP)
- Open Academy Meetup – Everything you wanted to know about the reform of the EU Pharmaceutical Legislation, Sep 28 (IMSP)
- RBD Echo New Therapies, October 5 (IW)
- OIF hybrid conference about transition, Oct 19 and 20 (IW and IMSP)
- ISCBH Winter Webinar, Dec 7 (IMSP)

ONLINE MEETINGS AND VIDEOCALLS INITIATED/HOSTED BY OIFE

For OIFE, keeping up to date with OI-related topics is key.

Networking and talking to people and organizations working in the field, is of great importance. Videocalls initiated by OIFE included stakeholders like GlobeReg, Metabolic Support UK, Global Pediatric Endocrinology and Diabetes (GPED), European Society of Endocrinology (ESE), Rare Revolution Magazine and various researchers and clinicians.

After the meeting with ESE we applied for associate membership with the umbrella.
ATTENDANCE AT EVENTS HOSTED BY OUR MEMBER ORGANIZATIONS

On August 25th we were invited to the 12th anniversary of the Spanish foundation Fundación Ahuce, which took place in their offices in Valencia, Spain. Before the celebration we were invited to a business lunch with representatives from Ahuce Foundation, dr. Ana Bueno, OIFE volunteer Maria Barbero and representatives from Mereo Biopharma.

OIFE was represented at the NFOI Adult Meeting, Sep 16th, where Ingunn gave an update from OIFE.

On May 27 Ingunn gave a talk at the online international African networking event hosted by the OIF Nigeria, which goal was to inspire a closer collaboration between groups and volunteers in Africa.

We also sent video greetings to the Establishing meeting of OI Panama and the Dutch anniversary (VOI).

OIFE CONTACT DATABASE

In addition to the list of subscribers and our industry list, OIFE keeps a database of more than 1300 professionals worldwide, that is mainly used to distribute the OIFE Magazine, invitations for scientific conferences and information about research grants. Due to several OIFE events ca 100 new researchers were added to our list during 2023.
COMMUNICATION & PR

As an organization committed to advancing awareness, support, and advocacy for the OI community, OIFE’s communication efforts play a pivotal role in shaping perceptions, fostering community engagement, and driving meaningful impact. Throughout the year, we prepare for key milestones, campaigns, and events that serve as important moments in OIFE’s ongoing mission.

Thanks to a growing number of dedicated volunteers, OIFE was represented on a record number of arenas in 2023.

When an OIFE representative attends a meeting or conference or when a new project is kicked off, we make an effort to post about it on OIFE social media and/or the website.

RARE DISEASE DAY CAMPAIGN 2023

The main objective of Rare Disease Day (last day of February every year) is to raise awareness amongst the public and decision-makers about rare diseases and their impact on people’s lives. It is a globally coordinated movement working towards equity in social opportunity, healthcare, and access to diagnosis and therapies for people living with a rare disease (PLWRD).

In the RDD 2023 campaign, OIFE focused on shedding light on neglected topics and areas within the rare disease - and OI community, aiming to generate greater awareness and understanding. Through this initiative, we sought to introduce the key themes that would be prominently featured in the upcoming OIFE Topical Meeting. Our goal was to ignite discussions, spark curiosity, and ultimately drive initiatives for further exploration and research.
Special attention was reserved for the topic pain in OI. Amidst our broader focus on neglected topics within the rare disease sphere, we recognized the urgent need to shine a spotlight on the unique challenges and experiences associated with pain’s impact and pain management in OI. Pain is an important and debilitating aspect of living with OI, yet it was relatively underexplored in both research and public awareness. Through our pain project and our campaign, we sought to elevate the voices of individuals living with OI, caregivers, and healthcare professionals, shedding light on the multifaceted nature of pain and its profound impact on quality of life.

The RDD 2023 campaign was successful in setting the stage the forthcoming OIFE Topical Meeting, making way for collaborative dialogue, knowledge exchange, and collective action.

WISHBONE DAY 2023

Every year the OI community eagerly anticipates the arrival of the 6th of May, a date marked by Wishbone Day celebrations. This annual event serves as a beacon of awareness, uniting individuals worldwide to shed light on the diverse experiences of those living with OI. Wishbone Day aims to be a platform to highlight the challenges faced by individuals with OI and explore opportunities and solutions for leading fulfilling, successful and joyful lives.

In 2023 OIFE had initially planned to use Wishbone Day as an opportunity to share preliminary findings from the IMPACT survey. However, it became evident that the research and analysis phases connected to the survey required additional time and would not have been available. Furthermore, the immense workload associated with organizing the upcoming OIFE Topical Meeting made it necessary to reallocate resources.

www.wishbonediday.com
International awareness day for Osteogenesis Imperfecta (OI)
Despite the absence of a dedicated campaign for Wishbone Day in 2023, the OIFE remained committed to the spirit of the occasion.

Instead of spearheading a centralized initiative, emphasis was placed on empowering national organizations within the OI community to lead their own awareness efforts.

OIFE actively shared posts from our members and individuals with OI. An online Zoom after party was hosted by OIFE on May 7th with online games and activities and the voting of the best photos and posts shared online for Wishbone Day.

**SOCIAL MEDIA**

OIFE continued its proactive engagement on social media platforms, leveraging them as dynamic channels to disseminate content, promote events and foster community interaction.

With a strategic focus on platforms like Facebook, Instagram, LinkedIn, and YouTube, the aim was to tailor our messaging as much as possible to resonate with diverse target audiences while amplifying the voices and stories within the OI community. Social media were used to actively share information, and to promote OIFE’s events, activities, campaigns, surveys etc. We also actively shared examples, news and stories from other stakeholders, with content we believe can be of interest to the larger community.

On Facebook and Instagram, our primary aim was to engage people with OI and the public, fostering a sense of community while raising awareness about OI-related issues.

By the end of 2023, our Facebook page had 4,187 followers, marking an increase of 387 followers from the previous year.

Similarly, on Instagram, we continued to grow our presence, ending the year with 1,114 followers, reflecting an increase of 304 followers.

Through visually engaging posts and stories with the #osteogenesisimperfecta, we created an inclusive and supportive online environment for individuals with OI, while also attracting interest from the broader public.
On LinkedIn, our focus shifted towards targeting OI professionals, researchers, and industry stakeholders, positioning ourselves as a trusted source of information and expertise within the field. With 935 followers by the end of 2023, a gain of 326 followers, our LinkedIn presence continued to strengthen, facilitate knowledge exchange and professional networking opportunities.

Our activity on X (formerly Twitter) saw a decrease in 2023. We had 1,402 followers, which was an increase of 63 followers. We however remained committed to keep a presence across all platforms.

On YouTube, we worked to expand our video content library, attracting 87 subscribers by the end of the year, a growth of 25 followers.

Through strategic engagement and collaboration, we will continue to harness the potential of these platforms to amplify the voices of OI organizations as well as of individuals with OI, drive meaningful change, and achieve our goals of better care and services for people with OI in all age groups.

OIFE MAGAZINE

We published four editions of OIFE Magazine in 2023. There were two special editions on the topics “OIFE around the world” and “Fracture risk & surgery in OI”. Target group includes professionals, people with OI, collaborators, and industry contacts – between 2000 and 2100 recipients.

The content ranges from news from OIFE, interviews with researchers, clinicians, OIFE volunteers, artists, activists and athletes with OI or just interesting people with a connection to OI.

Many of the articles are also published as blogposts on our website and shared on social media, something which increases the reach of the magazine substantially.

Quote from OI professional:

“I look forward to the OIFE Magazine. It serves as an excellent modality to keep to global OI community informed and connected. A true example of partnerships across all key stakeholders.”
OIFE WEBSITE

OIFE’s website has been regularly updated with a focus on OIFE-related activities and projects.

We published ca 85 blogposts in 2023 about events and projects as well as republishing articles from the OIFE Magazine.

The event calendar was also regularly updated with both community events and events relevant for clinicians and researchers working with OI.

We used our webpage actively to promote important events like the Topical Meeting and the OIFE Investigator Meeting.

DEVELOPING A BRAND IMAGE FOR OIFE & THE TOPICAL MEETING

Balancing Life with OI required a solid and cohesive brand image and OIFE’s communication team dedicated a lot of time to create materials and gadgets to be used onsite.

Recognizing the significance of visual and brand management, a dedicated logo was created for the event. By making most talks available on the OIFE YouTube channel, the meeting ensured broad accessibility, education, and advocacy within the OI community and beyond. In addition to the talks, we also produced two new videos for creating awareness about the OIFE – an after movie from the conference and a video called “What does OIFE mean to you?”

INTERNAL COMMUNICATION

Approximately 40 internal emails were sent to the OIFE representatives during 2023.

Instead of gathering a lot of content in long emails with a long list of links and attachment, we chose another strategy this time – and sent a larger number of emails with one or very few topics per email.
RESEARCH & DEVELOPMENT

PATIENT INVOLVEMENT

OIFE believes that all research projects affecting OI directly should have some kind of patient involvement. Patient involvement can happen in different ways and in different phases of a project. In addition to providing advice to the TOPAZ trial (run by academia), we provided advice from patient perspective to the following commercial stakeholders in 2023:

- Mereo Biopharma & Ultragenyx (Cosmic and Orbit)
- UCB & AMGEN (Romozosumab vs. Bisphosphonates)
- Sanofi (Poisel)
- BOOST Pharma (BoostB4 trial)
- Angitia Bio
- Azafaros
- Relation Therapeutics

OIFE has signed confidentiality agreements (CDAs) with several of these companies.

In 2023 we also had contact with the following industry stakeholders who are not directly working with OI: Kyowa Kirin International, PuREC, Pega Medical R&D, Alexion/AstraZeneca, Aparito, Putnam Associates, Semantic Hub, INNOSKEL and IPSEN.

ACCESS TO NEW AND INNOVATIVE TREATMENTS

There are an increasing number of new and potential treatments being investigated for OI at the moment. Some are in a preclinical phase (animal research) and some are in clinical trials. This includes Advanced Therapy Medicinal Products (ATMPs) and more common pharmaceutical products.

The European Medicines Agency (EMA) approves market access in Europe, but decisions about access and reimbursement are made in each and every country. We have therefore continued our effort to ensure that national organisations are knowledgeable on regulatory processes and health technology assessments. OIFE Leadership meeting 2023 included a dedicated talk about this.
We have also tried to collect and systematise patient experience data (PED) about the current situation for people with OI in initiatives like the IMPACT survey, Pain and OI survey and others. The data from these surveys can be used to document unmet needs, impact of the condition, patient priorities and value in future processes about approval and reimbursements.

Quote from Rare Disease stakeholder:

"The journey is not “just” about developing a therapy. it is about ensuring equitable access to that therapy."

RESEARCH PROJECTS WHERE OIFE WAS ACTIVELY INVOLVED

EJP RD CALL “NATURAL HISTORY STUDIES”

Via an announcement initiated by OIFE, we established a consortium of interested researchers who developed a project application about biomarkers and OI for the EJP RD call “Natural History Studies addressing unmet needs in Rare Diseases”. The consortium was led by MAB-member Antonella Forlino. In addition, we had 6 interested researchers from different European countries collaborating with OIFE as members of the consortium. The work started in 2022 and we had one meeting in 2023. Logistics were carried out by OIFE. Unfortunately, the project was not funded.

HR-PQCT WORKING GROUP OI

OIFE was represented with two people at the initial meeting of the HR-pCT Working Group on OI, which took place on October 5 on Zoom. The project is a consensus project on imaging of OI, initiated by ERN BOND and the Rizzoli Institute. The goal is to develop an overview of which clinics have access to Extreme CT (HRpQCT), compare measuring of bone through DEXA scans and HRpQCT and develop standardization recommendations. The work is led by dr. Enrico Schileo and around 25 researchers and patient representatives from Europe and US/Canada are involved.
REMEDI4ALL - THE MOI-A TRIAL (LOSARTAN)

OIFE was in 2023 represented by Stephanie Claeys in the EU-funded multidisciplinary consortium REMEDI4ALL, which focuses on repurposing of medicines. Alessandra Tolaccia (Italy) and Marina O’Callaghan (UK) were also involved. The projects consist of four demonstrator projects, where the MOI-A Trial investigating the “blood pressure drug” Losartan in OI is one of them. The patient involvement activities were led by EURORDIS supported by the UK non-profit Beacon. The project includes funding for 5 multi-stakeholder meetings on repurposing, where one will be OI specific.

THE OI VARIANT DATABASE

In 2023 OIFE (Taco) continued our volunteer work with helping the Amsterdam UMC to shorten the OI Variant database backlog and find new ways of recruiting more professionals to provide data. The team has now processed most of the remaining work in the previous years: 2019, 2020, 2022 are completed. Only the years 2021 and 2023 has some remaining work.

CO-AUTHORSHIP ON AND ABSTRACTS AND SCIENTIFIC ARTICLES

In 2023 OIFE contributed to a number of abstracts and scientific articles as co-authors. This included the IMPACT survey, The Saturn project, The IPSEN project and the care pathways project. See IMPACT survey for a list of relevant articles. The other articles included:

- Project SATURN – a Real-World Evidence Data Collaboration with Existing European Datasets in Osteogenesis Imperfecta to Support Future Therapies (submitted, awaiting final decision)
- Project SATURN – Meeting Patients, Regulatory, Health Technology Assessment and Payer Compliance in Real-World Evidence Data Collection (in final draft phase)
- Osteogenesis imperfecta: A study of the patient journey in 13 European countries (in final draft phase)
- “A Systematic Literature Review of the Impact and Measurement of Mobility Impairment in Rare Bone Diseases” (awaiting submission).

SUPPORT LETTERS, SURVEYS & HELP WITH RECRUITMENT

Research related support letters were provided to 3 researchers and two other stakeholders in 2023. In addition to our own survey on pain OIFE did not promote any other surveys in 2023. OIFE helped with recruitment in the project called "The Collaborative Production of “The Hospital of No Surprises”: An Animated Ethnodrama Designed with Children with Osteogenesis Imperfecta to Optimize their Surgical Experiences"; run by Shriners Hospital for Children in Canada.
PEER WORK

PEER GROUPS ON FACEBOOK

We have two peer groups for different target groups on Facebook one for adults who have OI themselves and one for young people with OI between 16 and 35. We don’t have a dedicated group for parents, but we guide parents to the American Facebook group for parents if needed.

FACEBOOK-GROUP “OIFE – OI & ADULT HEALTH”

OIFE’s actively moderated Facebook-group “OIFE – OI & adult health” is used by individuals to have peer support from other OI adults with similar experiences. The group increased from 1259 members to 1300 members in 2023. The members come from more than 75 countries.

The group was moderated by Ute Wallentin, Ingunn Westerheim, Julia Piniella, Rebecca Tvedt Skarberg and Taco van Welzenis. Through the group, we also receive feedback about what the health concerns of adults with OI are.

THE MOST ASKED ABOUT TOPICS IN 2023 INCLUDED:

1. Hearing impairment and surgeries (stapedectomies, CI etc)
2. Pain
3. Bone density & fractures (incl. non unions, stress fractures, surgery etc)
4. Pregnancy-related topics
5. Parathyroid problems and possible relation to OI
6. Medical treatment
7. Cardiopulmonary questions
8. Endocrinology
9. Mobility aids (incl. service dogs)
10. Dental problems and possible solutions (incl. implants)

Other topics included: blood pressure, weight loss, food supplements, mental health, finding shoes & clothes in small sizes, arthritis, rehabilitation, and practical advice (urinal, best chairs), post-operative care, aging with OI, menopause and its consequences.
FACEBOOK GROUP - OIFE YOUTH ONLY

The group is for people with OI between 16 and 35. There was very little activity in the group in 2023, however the group increased from 174 to 182 members. OIFE’s Youth Coordinators are moderators.

THE SOCIAL NETWORK

Maria Barbero and Ute Wallentin are OIFE’s social network coordinators and deal with incoming requests from individuals via e-mail, social media, and WhatsApp. Often requests come from countries without an OI-organization and can be anything from need for advice on how to find an OI-group, how to find competent care, the wish to emigrate to another country to requests for financial support and mobility equipment.

Sometimes we get requests from a person in a country with a functioning organization and in that case, we forward it to the local support OI-association or cooperate closely with them.

Most requests in 2023 came from parents or their representatives, fewer from OI adults. OIFE does not in any case provide financial support to individuals. But we give advice and peer support and help to find OI-groups or doctors with experience locally. We are still in need of more volunteers to help with replies to these requests.

In 2023 incoming requests came from all over the world, except from the bigger Asian countries. But we had requests from the US, the Philippines, various African countries and from inside Europe. We cannot help when someone asks for money or help to get Visa for emigration into other countries, but often we can provide basic information on OI and addresses of organizations, clinics and connect families from a certain country with others we know.
FINANCIAL STATUS

FINANCIAL REVIEW

The financial statement shows that OIFE reported a surplus of €16.357,29 for the year 2023.

INCOME
The total income for the year was € 196.588,13.

A large percentage of our overall income was for the purposes of our Topical Meeting and Pain Project. In total, 50 % of our annual income in 2023 came from pharmaceutical companies. The remainder came from a variety of sources such as grants, registration fees for events, membership fees and general donations.

We thank our supporters, sponsors and grant givers in 2023:

• European Joint Programme on Rare Diseases – € 30.000 project grant topical meeting
• Mereo Biopharma – unrestricted grant
• Ultragenyx – project grant topical meeting
• UCB – project grant topical meeting & pain project
• Alexion – sponsorship topical meeting
• Angitia Bio – donation topical meeting
• Azafaros – donation topical meeting
• Quince Therapeutics – donation topical meeting
• Orthopediatrics – donation general

Membership fees were slightly down in 2022. This is due to our fee level being dependent on individual organisations income and more member organisations requesting to pay a reduced fee.

The Board would like to thank all individuals and organisations who made donations to the OIFE during 2023.
**EXPENDITURE**

Expenditure for the year was €180,230,84.

As part of the grant funding mentioned above in relation to the topical meeting, we were able to outsource professional services such as the admin in planning the event, IT and AV support, and writing up of articles associated with the Pain Project linked to the Topical meeting.

The Board was increased from 5 persons to 7 persons at the AGM in 2022. This has meant an increase in travel costs associated with Board meetings as well as the general rise in travel costs as per inflation.

**FLYING OI EXPERTS**

The OIFE agreed to support two doctors from Venezuela and USA to fly to Nigeria as part of the flying OI Experts programme. These funds had not been used since pre-covid. We also had representation at various medical conferences where we funded travel for Medical Advisory Board Members.

**SUMMARY**

The Board are satisfied with the financial position of the organisation and can confirm the accounts have been audited by SBB the accountancy firm in Belgium to ensure we meet all appropriate legislative requirements according to Belgian law.
SUMMARY AND THANK YOU!

The Board of OIFE regard these as the most important positive developments and challenges we faced in 2023:

POSITIVE DEVELOPMENTS

- Very successful topical meeting on Pain & OI.
- Well received pain and OI survey – almost 1100 responses.
- Large increase in income – grants from industry and EU, made outsourcing of work possible.
- Lots of interest in OI research. OIFE Investigator Meeting brought many new contacts.
- Increased awareness of aspects of OI, through dissemination of findings from IMPACT.
- Two successful campaigns created attention about OI and the OIFE.
- Large increase of followers on LinkedIn – with increased visibility among professionals.

CHALLENGES

- OIFE Drop-In sessions were not a big success with little interest from the community.
- Lack of time to prepare a complete strategy draft due to high workload from topical meeting.
- Facebook is increasingly complicated to use due to a lot of “noise”.
- Complex rules to adhere to as a Belgian nonprofit – costs a lot of time and money.
- Lack of enough diversity in funding
- More interaction with pharma – brings complexity and new legal questions.
- Time involved in sourcing patient experts, when input from patient perspective is needed.
- Distribution of 50 wheelchairs projects failed – many lessons learned.
THANK YOU

During 2023 many different people helped us represent OIFE in various meetings and contributed on various tasks.

This included elected volunteers, appointed volunteers, ad hoc volunteers, patient advocates in policy work and patient representatives in research & development.

We send a big thank you to all the formal and informal volunteers of the OIFE! And thank you to all collaborators, for wanting to work with us in such a constructive way.