The OIFE

- Osteogenesis Imperfecta Federation Europe (OIFE) is a European umbrella for organizations that deal with OI.
- Our international network includes 36 organizations worldwide:
  - 20 ordinary members – national groups in Europe
  - 12 associate members (partners) outside Europe
  - 4 supporting member organizations incl. Care4BrittleBones & Fundación AHUCE
- Our most important activities at the moment include:
  - Access to treatments
  - The IMPACT survey
  - Development of EuRR-Bone
  - Patient engagement in R&D
  - Potential pain project
- We represent our members in many different European forums and conferences (see logos)
THE iMPACT SURVEY

A collaboration between the OIFE, the OI Foundation & Mereo Biopharma
What is the Impact survey?

«Living with Osteogenesis Imperfecta: understanding experiences based on Community Insight & evidence»

The IMPACT Survey aims to generate a scientifically validated and published international set of data that will capture and document the impact that OI has on people and also on the economics of national healthcare systems. The collected aggregated and consolidated results for each individual country will also be given to the national OI member associations to use in local policy work.

- Collaboration project between OI Foundation, OIFE & Mereo Biopharma
- One time survey – NOT a registry/database
- Governed by a multistakeholder steering committee
- The data management committee consist of Dr Frank Rauch, Ingunn W (OIFE) & Tracy Hart (OIF)
- Purpose is to gather documentation & evidence that can be used by all stakeholders
  - Scientific documentation
  - Documentation which can be used in national health technology assessment & reimbursement discussions
  - Policy & advocacy:
    - If numbers are big enough, the national organizations will have access to national reports from survey – which can make policy work (as in advocating to access to services) easier on a national level.
- More information on www.oife.org/impact
Steering Committee

Chairman
Dr Frank Rauch

Wickenstones
(external company)

Katherine Bache
OIF

Tracy Hart
OIF

Dr Cathleen Raggio

Dr Michael Bober

Ingunn Westerheim
OIFE

Taco van Welzenis
OIFE

Dr Oliver Semler

Dr Lena Lande Wekre

Jessica Riviere
Ultragenyx

James Clancy
Mereo

Arun Mistry
Mereo

Claudine Woo
Ultragenyx
What do we need to know in this project?

Patient journey

- Understand the **Patient Journey** better:
  - Examples - with variations from country to country:
    - Which professionals do you see?
    - How often do you see your GP?
    - Do you have access to specialists/centres of expertise?
    - Do you have regular follow-ups?
    - Do you have access to genetic testing?
    - Which treatments are available in your country?
What do we need to know in this project?

Impact of OI on QoL

- What does OI mean for children and adults beyond fractures?
- How does it affect Quality of Life?
- Example:
  - if a treatment does not reduce fractures, but significantly reduces pain and fatigue, it’s important to have data on pain and fatigue
  - What about other issues? Breathing, blood pressure, hearing? Etc...
What do we need to know in this project?

Economic impact

- What is the **economic impact** of OI on patients, caregivers, families?
  - What are the direct health related costs (such as hospital visits, surgery, medication, medical equipment like rods etc)?
  - What are the indirect costs (such as mobility aids, adaptations, assistance, loss of income, family costs etc)
  - And who pays for it? Are the expenses covered?
- **Why do we need to know** about costs?
  - Because the authorities approving drugs (also for rare conditions) puts great emphasis on cost/benefit when new treatments and advanced therapies (ex: stem cells and gene therapies) are up for approval.
  - Which costs can the larger society save if you get X % better from this medication? Can people with OI work more? They need less health care services?
  - Usually the national authorities are only interested in direct costs, but some countries and agencies put more emphasis on the bigger picture (incl. indirect costs)
HOW DO WE WANT TO DO IT?

- **Development of content**
  - collaboration between OIF/OIFE, clinicians and company

- **On-line global survey**
  - available to all in multiple languages

- **Awareness and recruitment – We need to work together!**
  - OIFE & OIF will spread the word to our networks to increase impact
  - OIFE member organizations can give regional support – and recruit people to answer the survey
  - Clinicians can help spread awareness and encourage patients to take part
  - How do we solve the problem of survey fatigue? By starting early with creating awareness about “what’s in it for me?”

- **External company Wickenstones has been hired to**
  - Set up systems, create documentation
  - Centralised anonymous hub for data receipt
  - Data analysis
POLICY WORK & ADVOCACY

With better data – the organizations can better advocate for improved services & treatments for children and adults with OI – internationally and nationally.
### Literature search has been done

- Literature review to identify evidence gaps
  - Literature search in 8 databases and grey literature (data from 1995 – 2020)
  - Whilst a sizeable body of literature describing bone conditions, anthropometric measurements, and the impact of OI on bone health and health-related quality of life (HRQoL) was retrieved, a significant number of data gaps were identified.

### Literature review to identify evidence gaps

### Survey design, build out and recruitment campaign:

- This step includes drafting and piloting the survey, translation into multiple languages, and co-creation of recruitment materials (with OI community reps)

### Launch of the survey

- Launch to take place end of June.
- Will need the help of ALL the organizations in June, July and August
- Survey will close September 10th 2021