

QUALITYOFLIFE4OI PROGRAMME

22nd – 25th NOVEMBER 2019 – AMSTERDAM

Status June 2019

FRIDAY 22 NOVEMBER

Time	Title	Speaker(s)	What to expect
12.00 – 20.00	Reception desk open		
13.00 – 14.30	Pre-Kick off for OI Community	OIFE, OIF, Care4BrittleBones	Get connected, consider your own role in the conference and how to make it valuable for you and others
15.00 - 15.40	Opening of the conference	Graham Marshall (chairman Care4BrittleBones), Frank Rauch (Montreal, Canada. Chairman of the Conference), Cindy Wan (President of Dutch OI Association VOI)	Warm welcome, orientation, interaction
15.40 – 16.10	Welcome message honorary guest		
16.10 – 16.30	Welcome message other steering committee members	Luca Sangiorgi (ERN-BOND) / BBDC (tbc)	
16.30 – 17.00	The changing role of patients	Ingunn Westerheim (OIFE, Norway) and Tracy Hart (OIF, USA)	Historic perspective, status quo and future trends.
17.00 – 17-45	Theater / interactive introduction to the themes of the conference	Work in progress	
17.45 – 18.15	Key note speech: Fresh perspectives on patient priorities in research and patient views on research practices	Nick Bishop (Sheffield, UK)	Key Note speakers sharing reflections and thought provoking ideas that will be discussed throughout the conference
18.15 – 18.30	Practical announcements	Dagmar Mekking (Care4BrittleBones)	
18.30 – 19.30	Cheese & Wine welcome reception	All	Included in conference fee <ul style="list-style-type: none"> Reconnecting with colleagues, socializing and enjoying the start of 4 special days.

SATURDAY 23 NOVEMBER - OVERALL

Time	Title	Speaker(s)	What to expect
8.00 - 8.45	Early Bird Sessions (optional)	Work in progress	5 interactive and energizing workshops on a broad variety of topics
9.00 – 10.00	Plenary Kick off	Frank Rauch , chair of the Conference	Orientation / Focus for the day
10.00 – 11.00	Deep-Dive Sessions (part 1 of 4,5 hour parallel sessions)	Workshop 1: Medical treatment options Workshop 2: OI Surgery: A roadmap to optimal functional outcomes and patient satisfaction Workshop 3: Psychosocial aspects of OI Workshop 4: Diagnosis of OI Workshop 5: Patient Empowerment Workshop	
11.00 – 11.30	COFFEE BREAK		
11.30 – 13.00	Deep-Dive Sessions (part 2 of 4,5 hour parallel sessions)	continuation	
13.00 – 13:45	LUNCH		
13.45 – 14.30	POSTER SESSIONS incl guided tour for OI-Community (part 1)		
14.30 – 16.30	Deep-Dive Sessions (part 3 of 4,5 hour parallel sessions)	continuation	
16.30 – 17.00	COFFEE BREAK		
17.00 – 19.00	Rare Bones Diseases What can we learn across rare bone diseases	Chair: Coen Netelenbos , Mareliese Eekhoff (both Amsterdam, NL), • XLH: Carola Zillikens (Rotterdam, NL)	After an introduction the session will raise disease awareness for - XLH - HPP - FOP zooming in on challenges to QoL, all available treatment options and outcome measures/clinical endpoints. The session closes with a Panel discussion about what we can learn from each other and synergies that may exist across the rare bone disease space.
20h	Amsterdam by night - boat tour through the canals of Amsterdam (300 places available. Accessible boats) – included in ticket price		

SATURDAY 23 NOVEMBER - DEEP DIVE SESSIONS

Deep Dive Session 1: Medical treatment options

Purpose: to provide an overview of all key medical treatment options for OI children and adults, discuss questions arising in clinical practice and how to measure treatment effectiveness

Led by **Frank Rauch** (Montreal, Canada), **Oliver Semler** (Cologne, Germany), **Anton Franken** (Zwolle, NL), **Lieve Michiels** (Belgium)

Time	Title	Presenter	What to expect
10.00 – 10.05	Introduction	Frank Rauch (Montreal, Canada)	Overview workshop, objective, process
10.05 – 10.35	Bisphosphonates in Children – how are we doing it?	Frank Rauch (Montreal, Canada)	Discuss bisphosphonate treatment approaches in children with OI 1. standard of good clinical care incl “do’s and don’ts” + key questions prepared upfront. 2. measuring effectiveness of treatment
10.35 – 11.00	Bisphosphonates in Adults – how are we doing it?	Anton Franken (Zwolle, NL)	Discuss bisphosphonate treatment approaches in adults with OI Same as for children
11.00 -11.30	COFFEE BREAK		
11.30- 11.55	Anabolic drugs and Antisclerostin– what do we know?	<i>Work in progress</i>	The landscape of anabolic drugs. Discussion of use of sclerostin antibodies in OI
11.55-12.20	TOPaZ - trial	Stuart Ralston (Edinburgh, UK)	Interim report on OI Trial Adults (400 patients); discussion of use of teriparatide in OI
12.20-12.45	Other short acting drugs – where are we at?	Oliver Semler (Cologne, Germany)	Discuss denosumab, TGFbeta and other drugs in the pipeline
12.45-13.00	Calcium, vitamin D, vitamin K and other supplements – what is the evidence?	tbc	Discuss need for supplements in OI
13.00 – 14.30	LUNCH + POSTERS		
14.30-14.55	New Drugs – how to study them?	Nick Bishop (Sheffield, UK)	Discuss design for new drug studies in OI

14.55-15.20	Stem cell treatment	Cecilia Götherström (Stockholm, Sweden)	Discuss approaches for stem cell treatment
15.20 – 15.40	Towards a therapy for OI - CRISPR – CAS 9	Fleur van Dijk (London, UK)	Discuss approaches using CRISPR-CAS technology in OI
15.40-16.30	<p>Discussion: What is “good medical therapy” for OI?</p> <ul style="list-style-type: none"> - Overall: what do we see as the standard of good clinical care (evidence + expert opinion). - What is NOT recommended? 	Chair: Frank Rauch (All speakers of the workshop)	<p>Discuss the overall “state of play” today, distill the consensus for good clinical care based on both: evidence and expert opinion.</p> <p>Try to tease out the areas of “too little, too much, problematic use, dissenting views, questionable practices” to help those.</p> <p>Future trends</p>

Deep Dive Session 2: OI: Roadmap to surgery for optimal functional outcomes and patient satisfaction

Purpose: to discuss all aspects required before, during and after OI surgery to deliver optimal functional outcomes and patient satisfaction. The workshop focusses on planned surgeries of upper and lower extremities and spine, taking into account the patient perspective on priorities, a broad variety of treatment options, outcome parameters, the role of rehabilitation pre- and post-surgery, the applicability across different healthcare systems and cultures, infrastructure and skill levels of surgeons and the joint decision making with the patient.

Led by **Ralph Sackers** (Utrecht, NL), **Richard Kruse** (Wilmington Delaware, USA), **Guus Janus** (Zwolle, NL), **Reggie Hamdy** (Montreal, Canada), **Emily Lecker** (Montreal, Canada), **Kathleen Montpetit** (retired, Montreal, Canada), **Leonardo Panzeri/Simona Paveri** (Italy), **Marjolein Verhoef** (Utrecht, NL)

Time	Title	Speaker(s)	What to expect
10-10.30	Introduction	Ralph Sackers (Utrecht, NL)	Introduction to the workshop agenda and overall flow of the workshop. Introduction to the Roadmap concept
10.30–10.45	Patients’ view on surgical procedures – what works and what doesn’t	Leonardo Panzeri/ Simona Paveri (Italy, ASITOI)	Patients view and needs/checklist in relation to planned surgery
10.45–11.00	Recommended methods to include the view of the (family of the) patient	Emily Lecker (Montreal, Canada)	Robust ways to ensure the patient view is understood and factored into the decision
11.00–11.30	COFFEE BREAK		

11.30-12.00	Which outcome to achieve for spine deformities and how to achieve it?	Jean Ouellet (Montreal, Canada)	Roadmap/checklist for surgical indications in spine surgery
12.00-12.20	CHILDREN: Which outcome to achieve for lower extremities in children and how to achieve it?	Reggie Hamdy (Montreal, Canada)	Roadmap/checklist for surgical indications in lower extremity surgery
12.20-12.30	ADULTS: Which outcome to achieve for lower extremities in adults and how to achieve it?	Guus Janus (Zwolle, Netherlands)	Roadmap/checklist for surgical indications in lower extremity surgery
12.30-12.50	CHILDREN: Which outcomes to achieve for upper extremities in children and how to achieve it?	Thomas Wirth (Stuttgart, Germany)	Roadmap/checklist for surgical indications in upper extremity surgery
12.50-13.00	ADULTS: Which outcomes to achieve for upper extremities in adults and how to achieve it?	Guus Janus (Zwolle, Netherlands)	Roadmap/checklist for surgical indications in upper extremity surgery
13.00-14.30	LUNCH + POSTERS		
14.30-14.50	How to create the feed-back loop to the decision making process?	Kathleen Montpetit (Montreal, Canada)	Methodology of registration and self-learning with regard to decision making in previous indications for surgery
14.50-15.10	A generic roadmap for surgery for all cultures and gradations of wealth!	Richard Kruse (Delaware, USA)	How to create good care with regard to different needs and different infrastructures and financial restrictions in different cultures and societies.
15.10-15.30	The decision making conversation (including decisions on multiple surgeries)	<i>Work in progress</i>	How to listen, talk, discuss and “contract” with each other (HCP and patient) for optimal outcomes
15.30–15.50	Recommended principles and approaches to generate reproducible outcomes	Marjolein Verhoef (Utrecht, Netherlands)	Summarizing concepts, tools and processes recommended across the roadmap
15.50-16.30	Roadmap to surgery in OI: production of document/paper	Chair: Ralph Sackers (+ all speakers of the workshop)	Deepening insights / reflecting on obstacles Discussing behavior changes required to integrate this into clinical practice and OI patient preparation for surgery Asking for support to finalise and then make use of the Roadmap

Deep Dive Session 3: “Living with OI” – understanding and addressing the nonphysical aspects of OI.

Purpose: to discuss the latest insight regarding the key “nonphysical aspects of living with OI and how to support them effectively.

Led by **Kara Ayers** (Cincinnati, USA), **Claire Hill** (Sheffield, UK), **Ute Wallentin** (Germany)

Time	Title	Speaker(s)	What to expect
10:00-10.30	Introduction	Kara Ayers (Cincinnati, USA)	Overview of the workshop agenda and psychosocial network for knowledge sharing
10:30-11:00	Opening Panel: Psychosocial Aspects of Children and Adults with OI	Iris van Wijk (Utrecht, NL)	Introduce general themes and aspects of psychosocial functioning impacted by OI
11:00-11.30	COFFEE BREAK		
11:30-12:00	Measuring Quality of Life in Children and Adolescents with OI	Claire Hill (Sheffield, UK)	Describe process and purpose of a disease-specific QoL measure and its use in measuring QoL in children. Update attendees on Key4OI efforts related to measuring QoL.
12:00-12:30	Measuring Quality of Life in Adults with OI	<i>Work in progress</i>	Describe use of PROMIS measure and compare/contrast to use of disease-specific measures.
12.30-13:00	Discussion	Ute Wallentin (Germany, OIFE)	Space for reflection and discussion, moderated by Ute Wallentin
13.00-14.30	LUNCH + POSTERS		
14:30-15:30	Rapid 5’s: (1) Fatigue/Sleep, (2) Pain (3) Mental Health	(1) Arjan Harsevoort (Zwolle, NL), Heidi Arponen (Helsinki, Finland) (2) Nicole Lindenberg (Regensburg, Germany) (3) Argerie Tsimikalis (Montreal, Canada)	Interactive session with 3 booths with a 5 minute presentation followed by 10 minutes of discussion. Booth 1: Fatigue Booth 2: Pain Booth 3: Mental Health
15:30-16:30	Establishing Standards to Improve the Psychosocial Care of Children and Adults with OI	Chair: Kara Ayers (+ speakers involved throughout the workshop)	The session summarizes the presentations of the workshop and discusses what should be psychosocial standards (must) and guidelines (should) in treatment of children/adults with OI.

Deep Dive Session 4: Diagnosis of OI

Purpose: To understand the diagnostic modalities in OI (past, present and future trends). To explore the impact of early and correct diagnosis for QoL with OI and propose a standard of good diagnosis for clinical practice

Led by: **Meena Balasubramanian** (Sheffield, UK), **Valerie Cormier Daire** (Paris, France), **Peter Byers** (Washington, USA), **Lida Zhytnik** (Estonia)

Time	Title	Speaker(s)	What to expect
10:00-10:30	Introduction to workshop	Meena Balasubramanian (Sheffield, UK), Valerie Cormier Daire (Paris, France)	Setting the scene and expectations. Presenting a diagnostic work up. Molecular diagnosis vs clinical diagnosis
10:30-11:00	Historical Perspectives in OI diagnosis	Peter Byers (Seattle, USA)	What used to be diagnostic models in years gone by and is there still a role for these?
11.00–11.30	COFEE BREAK		
11:30-11:50	The genetic landscape of OI	Dimitra Micha (Amsterdam, NL)	From unclassified variant to confirmed OI causing variant
11:50-12:10	An OI (& professional's) perspective	Lida Zhytnik (Tartu, Estonia)	What are the patients questions / issues in relation to Diagnosis and QoL?
12:10–13.00	Diagnosis and QoL	<i>Work in progress</i>	Exploring the impact diagnosis can have on QoL
13:00-14:30	LUNCH & POSTERS		
14:30-16:00	Guideline for Good clinical practice: Diagnosis for OI	Chair: Meena Balasubramanian (Sheffield, UK), Valerie Cormier Daire (Paris, France) + all presenters of the workshop	Seeking consensus on best model for first line diagnosis in OI - Who, what, how (affordable, available everywhere, effective, quick and a good experience for the patient-family)

Deep Dive Session 5: Patient Empowerment Workshop

Purpose: for people in the OI community to learn and discuss about research and health care improvement!

Led by OIFE/OIF

Details to be announced shortly.

SUNDAY 24 NOVEMBER

Time	Title	Speaker(s)	What to expect
8.00 – 8.45	Early Bird Sessions (optional)	Work in progress	5 interactive and energizing workshops on a broad variety of topics
9.00 – 11.00	Summary of Deep – Dive Sessions	Chair: Graham Marshall (Chairperson Care4BrittleBones);	Report out - presentations of the chairpersons of the Deep-Dive workshops. Q&As
11:00 – 11:30	COFFEE BREAK		Market stands of MDT – MDT-teams around the world have the possibility to present themselves
11:30 – 11:50	Welcome and introduction of theme “Multidisciplinary treatment of OI”	Chair: Lena Lande Wekre (Oslo, Norway), Oliver Semler (Cologne, Germany) Moderator: Graham Marshall	Purpose is to focus on OI as a systemic disease and create a shared view on standards for good clinical care and follow up routines
11:50 – 12:30	Patient perspectives on the need for MDT	OI patients	Provide realistic insight into MDT challenges today dealing with different types of OI in different phases of life. Situations that typically require a MDT approach (e.g. pain, orthopedic intervention etc)
12:30 – 13.00	OI as a systemic disease	Work in progress	Presenting all aspects of OI, bone and non-bone aspects of OI
13.00 – 13.45	LUNCH		
13.45 – 14.30	POSTER SESSION incl. guided tour for OI-Community (part 2)		
14:30 – 16:00	Various approaches to MDT	Work in progress	Presenting 4 different MDT models. Discussing the minimum requirements and what “excellent MDT” could look like, challenges and solutions.
16:00 – 16.30	COFFEE BREAK & interactive session		Market stands of MDT – MDT-teams around the world have the possibility to present themselves
16.30 – 16.45	Introduction afternoon session	Chair: Lena Lande Wekre (Oslo, Norway), Oliver Semler (Cologne, Germany)	
16.45 – 17.15	ERN-BOND Project “Guidelines for good clinical care”	ERN BOND representative (tbc)	Guidelines for good clinical care of OI Project update and next steps.

17.15 – 17.45	Project «Key4OI»	Project team representative	Introduction to outcome measurement set «Key4OI», Project update and next steps
17.45 – 18.30	Panel discussion: “How to bring multidisciplinary treatment for OI to the next level?”	Moderator/ Speakers of the day	Discussion, reflection, provocative fresh thoughts and “making it practical”
18:30 – 19:30	Free time		
19.30 – beyond	Festive “Unbreakable Spirit” Conference Dinner including performances (included in conference fee)		

MONDAY 25 NOVEMBER

Time	Title	Speaker(s)	What to expect
9.00 – 9.45	Recognition of special achievements	Award winner(s)	Celebrating special achievements to advance Quality of life 4 OI (Awards / Grants). Group foto Presentation of Conference Choice award winner
9.45 – 10.15	EMA’s perspective – trends for using real life data for measuring patient outcomes	EMA speaker (confirmed)	Presentation & discussion
10.15 – 11.15	Panel discussion <topic under discussion>	Selected conference speakers	Panel to consist of ERN-BOND (tbc), BBDC (tbc), patient associations, pharma, EMA (tbc). Moderator: Graham Marshall (Chairman Care4BrittleBones)
11.15-11.45	Key take aways	Steering Committee members	Personalization. Key take aways and actions steering committee and audience.
11.45 – 12.00	Closing Ceremony	All	
From 12.00	GRAB AND GO LUNCH	All	