Supporter Profiles

Alexion

www.alexion.com

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialisation of life-changing therapies. Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies – Kanuma™ (sebelipase alfa), for patients with lysosomal acid lipase deficiency (LAL-D), and Strensiq® (asfotase alfa), for patients with hypophosphatasia (HPP). Alexion also developed and commercialises Soliris® (eculizumab), an approved terminal complement inhibitor to treat paroxysmal nocturnal haemoglobinuria (PNH), atypical haemolytic uremic syndrome (aHUS) and anti-acetylcholine receptor (AchR) antibody-positive generalised myasthenia gravis (gMG).

Ascendis

www.ascendispharma.com

Ascendis Pharma is applying its innovative TransCon™ technologies to build a leading, fully integrated biopharmaceutical company focused on making a meaningful difference in patients' lives.

Guided by our core values of patients, science and passion, we utilize our technology platform to create new and potentially best-in-class therapies. We currently have a pipeline of three independent, rare disease endocrinology product candidates in clinical development. We have also established oncology as our second therapeutic area of focus.

BioMarin

www.biomarin.com

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. Approved products include the first and only therapies for PKU, LEMS, MPS I, MPS VI, MPS IVA, and CLN2 disease. Clinical development programs include investigational therapies for Hemophilia A, Achondroplasia, MPS IIIB, Friedreich's Ataxia and other rare diseases.

Clementia

www.clementiapharma.com

Clementia, an Ipsen Company, is innovating treatments for people with ultra-rare bone disorders and other diseases with high medical need. The company is preparing for a 2019 new drug application (NDA) submission to the FDA to seek approval of its lead product candidate, palovarotene, a novel RARγ agonist, for the prevention of heterotopic ossification (HO) associated with flare up symptoms in adults and children with fibrodysplasia ossificans progressiva (FOP). The ongoing Phase 3 MOVE Trial is evaluating an additional dosing regimen of palovarotene for the treatment of FOP. Palovarotene is also in a Phase 2 trial, the MO-Ped Trial, for the treatment of multiple osteochondromas (MO, also known as multiple hereditary exostoses, or MHE). In addition, Clementia has commenced a Phase 1 trial for an eye drop formulation of palovarotene for the potential treatment of dry eye disease . For more information please visit www.clementiapharma.com, or www.ipsen.com.

European Calcified Tissue Society (ECTS)

www.ectsoc.org

The European Calcified Tissue Society (ECTS) is the major organisation in Europe for researchers and clinicians working in the field of calcified tissues and related fields. For over 50 years the Society has acted as a forum to promote the highest levels of knowledge, research and education through its annual meetings, training courses and grants and awards. Membership of the ECTS is open to anyone working in the field at whatever stage in their career. Come and meet us during ICCBH at our booth.

Inozyme

www.inozyme.com

Inozyme Pharma is a biotechnology company committed to developing novel therapeutics for the treatment of rare, life-threatening and devastating disorders of mineralization. The company was founded in 2017 with technology licensed from Yale University.

Inozyme's lead program, INZ-701, is an enzyme replacement therapy in preclinical development and is indicated for treatment of ENPP1 Deficiency. ENPP1 Deficiency manifests as either generalized arterial calcification of infancy (GACI) type 1 or autosomal recessive hypophosphatemic rickets type 2 (ARHR2). ENPP1 Deficiency is a rare, devastating disease resulting from decreased levels of serum PPi. PPi is essential for preventing harmful soft tissue calcification and for regulating normal bone mineralization. ENPP1 enzyme replacement therapy has proven to be successful in normalizing serum PPi, increasing survival and preventing pathogenic calcification in the animal models of ENPP1 Deficiency.

Internis

www.internispharma.com

Founded in 2010, Internis is a UK speciality pharmaceutical company engaged in the development and commercialisation of highly effective and innovative medicines currently aimed at the treatment and prevention

of a range of common bone disorders, such as osteoporosis and vitamin D deficiency.

Internis has grown rapidly in the past 4 years and is still expanding. It is positioned now as one of the UK's fastest growing pharmaceutical companies, focusing on niche areas where there is an unmet medical need.

Internis recognises the important work of the Royal Osteoporosis Society and is committed to supporting this through the bone health partnership.

Kyowa Kirin International

www.kyowa-kirin.com

Kyowa Kirin International plc is a rapidly growing specialty pharmaceutical company focused on innovative medicines in rare diseases and therapeutic areas with high medical needs. Kyowa Kirin International has business operations throughout Europe and the United States.

Join us for our interactive satellite symposium chaired by **Prof. Wolfgang Hölger** *(Johannes Kepler University Linz, Austria)* '**X-Linked Hypophosphataemia (XLH): Old World, New Horizons**' on Saturday 22 June 2019, 17:15–18:45.

- **Management of XLH in the old world: advancing understanding from diagnosis to treatment**
  Prof. Agnès Linglart, *University of Paris-Sud, France*
- **New expectations for managing XLH: from clinical development to real practice**
  Dr Moira S Cheung, *Guy's and St Thomas' Trust, London, UK* and **Prof. Wolfgang Högler**, *Johannes Kepler University Linz, Austria*
- **Future horizons for a new generation of children with XLH**
  Dr Leanne Ward, *University of Ottawa, Canada*

Come and find out more by visiting the Kyowa Kirin stand (no. 1).

Mereo

www.mereobiopharma.com

Mereo is a UK-based international biopharmaceutical company focused on developing innovative treatments in the field of rare diseases; and to bringing these to patients in a timely and sustainable way. Our mission is focussed on providing new therapies to patients with chronically debilitating life-limiting rare diseases that have few, if any, other treatment options. Our lead programme is a potential treatment for Osteogenesis Imperfecta. Working with the OI community, we hope to be able to advance our potential therapy forward in the most effective and meaningful way for all stakeholders. Mereo's team of dedicated and passionate experts in their respective fields believe that, together, our internal expertise combined with our network of external resources, will enable us to rapidly progress our key programmes into late-stage development and the planned subsequent commercialisation and availability. We thank patients, physicians and all those involved in the diagnosis, treatment and care of OI, for their support and encouragement.

For more information, please contact us on hello@mereobiopharma.com.

Novo Nordisk

www.novonordisk.com

Novo Nordisk is a global healthcare company with more than 95 years of innovation and leadership in diabetes care. This heritage has given us experience and capabilities that also enable us to help people defeat obesity, haemophilia, growth disorders and other serious chronic diseases. Headquartered in Denmark, Novo Nordisk employs approximately 43,200 people in 80 countries and markets its products in more than 170 countries.

Regeneron

www.regeneron.com

Regeneron is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to seven FDA-approved treatments and numerous product candidates in development, all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, musculoskeletal diseases, infectious diseases and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*® technologies, such as *VelocImmune*® which produces optimized fully-human antibodies, and ambitious research initiatives such as the Regeneron Genetics Center, which is conducting one of the largest genetics sequencing efforts in the world.

Stratec Medizintechnik / Novotec Medical

www.stratec-med.com

www.novotecmedical.de

Stratec Medizintechnik is the world's most successful producer of pQCT-based bone densitometry scanners for clinical and preclinical applications. Unlike in 2-dimensional absorptiometric machines pQCT derived density measures are independent of bone size. Therefore pQCT can be

applied to all age groups and body sizes. Additionally geometrical properties of bone and muscle parameters can be analysed which allows a detailed diagnosis and the differentiation of disuse osteopenia from true osteoporosis.

Novotec Medical is manufacturer of Galileo vibration training devices for muscle stimulation and of Leonardo motion analysis systems (mechanography). Due to the side alternating technology a natural movement similar to human gait is employed. Several studies showed the benefits in the improvement of mobility of chronically ill children.

TamiRNA

www.tamirna.com

TAmiRNA is a biotech company specialized in the discovery and validation of microRNA biomarkers. Our mission is to provide minimal-invasive, fast and robust biomarkers to accelerate drug development, improve diagnosis and prognosis of disease, and support treatment decisions.

TAmiRNA's osteomiR™ test is a biomarker signature for bone quality, bone disease and fracture risk, based on blood-circulating microRNAs. Moreover, TAmiRNA offers a broad range of high-quality RNA analytical services, with focus on quantitative microRNA analysis in cells, tissues, extracellular vesicles and biofluids.

Therachon

www.therachon.com

Therachon is developing treatments for rare conditions with unmet medical needs. We are committed to fostering a community rigorous about science and passionate about transforming patient lives. Our lead pipeline candidate, TA-46, is a novel protein therapy in development for achondroplasia, the most common form of disproportionate short stature.

UCB

www.ucb.com

UCB, Brussels, Belgium (www.ucb.com) is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions to transform the lives of people living with severe diseases of the immune system or of the central nervous system. With 7,500 people in approximately 40 countries, the company generated revenue of €4.6 billion in 2018. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

Visiana

www.visiana.com

Visiana develops and markets BoneXpert, a method for automated bone age and radiogrammetry. BoneXpert is thoroughly validated and is already used in >150 hospitals and clinics across Europe, including some of Europe's biggest children's hospitals. Come visit our booth to hear about the possibility of a free trial in your hospital.

BoneXpert is also a powerful research tool, enabling quick analysis of 100s or 1000s of X-rays. BoneXpert measures bone age according to GP, TW2, TW3 and other bone age scales. We also measure a Bone Health Index (BHI), which is an expression of the relative thickness of metacarpal cortical bone.

Finally, we have developed an improved adult height prediction formula to replace the Bayley-Pinneau method.