NORDIC
RARE DISEASE
SUMMIT 2020

INVITATION

The challenges we are facing today within rare diseases are complex and extensive. To understand and overcome these we need to work together and share knowledge across countries, across expert groups and consult people who are living with rare diseases.

The Nordic Rare Disease Summit will be an arena for knowledge sharing and facilitate dialogue and discussions between clinicians, patient representatives and politicians.
At the summit you can meet leading experts within rare diseases from all Nordic countries, who will present new findings, share best practice and participate in discussions on how to elevate the area of rare diseases in order to support and empower people living with rare diseases.

The Summit will result in a shared commitment and call for action in a 'Copenhagen Roadmap for Rare Diseases', and throughout the day, you will have the opportunity to visit the Rare Disease Arena, where a broad range of best-practice cases, insights and projects from all Nordic countries, will be displayed by patient organisations and key actors within rare diseases. The summit is organized by Takeda and hosted in collaboration with Rare Diseases Denmark (Sjældne Diagnoser).

We hope to see you in Copenhagen!

Best Regards,

Ingeborg Rossebø Borgheim
Head of Nordics,
Takeda

Birthe Byskov Holm
President,
Rare Diseases Denmark

TIME AND PLACE
Date: March 13, 2020, 09.30-16.50
Place: Hangar 145, Vilhelm Lauritzens Alle 1, 2770 Kastrup, Denmark
(Shuttlebuses will be provided from Copenhagen Airport appx. 5 min drive)

PARTICIPATION
Free participation, registration required! Register for the summit HERE
Please RSVP by
Wednesday, February 26, 2020

QUESTIONS?
For more information please contact Charlotte Engel Møller, Senior Coordinator, Takeda Denmark:
Charlotte-Engel.Moller@takeda.com

Sjældne Diagnoser

Takeda

C-APROM/DK//0873

AGENDA

09:30 - 10:00 REGISTRATION & LIGHT BREAKFAST

10:00 - 10:35 WELCOME

Giles Platford, President Europe and Canada Business Unit, Takeda and Birthe Byskov Holm, Chair, Rare Diseases Denmark

Opening Address (video) by HRH Crown Princess Mary, Patron of Rare Diseases Denmark

10:35 - 12:10 THEME I: DIAGNOSTIC DELAY

Timely diagnosis and why it matters, by Arvid Heiberg, Prof. (em), Department of Medical genetics, Oslo University hospital, Norway

The Global Commission on how to End the Diagnostic Odyssey for Children with a Rare Disease, by Mathias Ekman, Director Industry Solutions Executive for Health and Life Science at Microsoft, Western Europe

Innovative technologies and the future of diagnostics, Helena Kääriäinen, Research Professor, National Institute of Health and Welfare, Helsinki Finland

Panel debate: How to ensure early diagnosis in the Nordic countries?
Moderator: Arvid Heiberg, Prof. (em), Department of Medical genetics, Oslo University hospital, Norway
Panelists: Maria Montefusco, Chair, Rare Diseases Sweden. Camilla Sønderby, Global Head of Patient Value & Product Strategy, Takeda. Allan Meldgaard Lund, Chief Physician, Center for Inherited Metabolic Diseases, Rigshospitalet

12:10 - 13:00 LUNCH & VISIT TO THE RARE DISEASE ARENA

13:00 - 14:35 THEME II: PATIENT EMPOWERMENT

The importance of empowerment for patients, relatives and society, by Terkel Andersen, President of EURORDIS - Rare Diseases Europe

3 x 10-minute perspectives on empowerment:

- **Micro/Patient level:** Experiences of Web-based adaptation training for people with rare diseases, Sinikka Hiekkala Project Mgr, The Finnish Association of People with Physical Disabilities
- **Macro level:** Rare Diseases Helpline in a societal perspective, by Birthe Byskov Holm, President, Rare Diseases Denmark, member of SBONN
- **Policy level:** Equal opportunities for People Living with Rare Disease, by Helene Cederroth, Founder and President, Wilhelm Foundation

Panel debate: How do we facilitate and improve empowerment nationally and cross country?
Panelists: Terkel Andersen, President, EURORDIS; Ingunn Westerheim, snr advisor, The Norwegian Federation of Organisations of Disabled People, member of SBONN, Guðrún Helga Harðardótti, Family Therapist and CEO, Einstök Börn, member of SBONN, Monica Hedman, Patient representative, Rare Diseases Sweden

14:35 - 14:55 COFFEE BREAK

14:55 - 16:30

THEME III: PATIENTS' ACCESS TO INNOVATION

European recommendations for improving overall access to medicines for patients with rare diseases across the EU, by Yann Le Cam, Chief Executive Officer, EURORDIS - Rare Diseases Europe

How the Nordic countries align with nine European OPRH-VAL principles for assessment of medicines for rare diseases, by Lieven Annemans, Professor of health economics, Ghent University, Member of ORPH-VAL Working Group

Access, Innovation and sustainability from a Norwegian perspective, by Stig Slørdahl, CEO at RHA Central Norway, and leader of the Decision Forum RHA

Panel debate: How can we improve patient's access to innovation?
Moderator: Lieven Annemans, Professor of health economics, Ghent University, Member of ORPH-VAL Working Group
Panelists: Yann le Cam, CEO, EURORDIS, Ida Sofie Jensen, CEO, The Danish Association of the Pharmaceutical Industry (Lif), Karin Friis Bach, Chair of Health Committee and Board Member, Danish Regions, Stig Slørdahl, CEO at RHA Central Norway, and leader of the Decision Forum RHA, Giles Platford, President Europe and Canada Business Unit, Takeda

16:30 - 16:50

COLLABORATING TO IMPROVE CONDITIONS FOR PEOPLE WITH RARE DISEASES

Transition to 'Rare Disease Arena' booth area

Presentation of 'Copenhagen Roadmap for Rare Diseases'
Learnings and key messages uniting participants in the Nordic Rare Disease Summit behind common aspirations, by Birthe Byskov Holm, Chair, Rare Diseases Denmark and Ingeborg Borgheim, Head of Nordics, Takeda

Opportunity to explore 'Rare Disease Arena'

PARTNERS

Prominent organizations from across the Nordic countries and the wider Europe have joined as partners:
EURORDIS, a non-governmental patient-driven alliance of patient organizations representing 862 rare disease patient organizations in 70 countries, SBONN, a network of six umbrella patient organizations for rare diseases in five Nordic countries and The Danish Association of the Pharmaceutical Industry (Lif).

EURORDIS RARE DISEASES EUROPE

SBONN

Lif

The following organizations have endorsed the Nordic Rare Disease Summit:

efpia European Federation of Pharmaceutica Industries and Associations

EUCOPE European Confederation of Pharmaceutical Entrepreneurs AISBL

Danmarks Bløderforening

IML

Code of Conduct Both Takeda (funder and organizer) and Rare Diseases Denmark (co-host) stress that it is a precondition for the collaboration that all activities comply with The Danish Association of the Pharmaceutical Industry (Lif), Swedish Association of the Pharmaceutical Industry - LIF and EFPIA's Code of Practice on Relationships between The Pharmaceutical Industry and Patient Organizations, national and EU regulations governing interactions between the pharmaceutical industry and patient organizations and Rare Disease Denmark's ethical guidelines. The event in its current form and content has been pre-approved by The Danish Ethical Committee for the Pharmaceutical Industry (ENLI).
About Takeda Takeda is a patient-focused, values-based, R&D-driven global biopharmaceutical company committed to bringing Better Health and a Brighter Future to people worldwide. Our passion and pursuit of potentially life-changing treatments for patients are deeply rooted in over 230 years of distinguished history in Japan. We have presence in approximately 80 countries, with leading positions in Japan and the U.S., respectively the third and first largest pharmaceutical markets in the world. We will deliver highly innovative medicines and transformative care for more people globally. www.takeda.com
About Rare Diseases Denmark (Sjældne Diagnoser) Rare Diseases Denmark is a national alliance of 56 rare disease societies. Rare Diseases Denmark's work is based on volunteers and a minor professional secretariat, www.sjaeldnediagnoser.dk.

C-APROM/DK//0873