NORDIC
RARE DISEASE
SUMMIT 2021

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 a virtual 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 'Nordic Roadmap for Rare Diseases'. This Roadmap for Rare Diseases sums up key messages, discussions and new knowledge from the Nordic Rare Disease Summit 2021 - with an aspiration to provide policy guidance relevant for the Nordic countries, recommendations for future cooperation and a shared call for action. The summit is organized by Takeda and hosted in collaboration with Rare Diseases Denmark (Sjældne Diagnoser).

We hope you will join our first ever virtual Nordic Rare Disease Summit!

Best Regards,

Duarte Marchand
Head of Nordics,
Takeda

Birthe Byskov Holm
President,
Rare Diseases Denmark

TIME AND PLACE

Date: April 12th, 2021, 12:45 – 17:00
April 13th, 2021, 12:45 – 16:00
Place: Virtual meeting

QUESTIONS?

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

Sjældne Diagnoser

Takeda

AGENDA - Day 1

NORDIC RARE DISEASE SUMMIT 2021

12:45 – 17:00 MONDAY 12 April 2021

12:45 – 13:00 Registration for virtual meeting
Help Desk: +45 50 92 72 03 or +45 31 21 31 84

13:00 – 13:40 WELCOME


13:00 – 13:25 Giles Platford, President Europe and Canada Business Unit, Takeda and Birthe Byskov Holm, Chair, Rare Diseases Denmark

13:25 – 13:35 **Introduction to program** by Moderator Line Friis Frederiksen, biologist, science journalist

13:35 – 13:40 **Opening Address (video)** by HRH Crown Princess Mary, Patron of Rare Diseases Denmark

13:40 – 15:10 THEME I: DIAGNOSTIC DELAY

13:40 – 13:55 **Timely diagnosis and why it matters,** by Arvid Heiberg, Prof. (em) Department of Medical genetics, Oslo University hospital, Norway

13:55 – 14:10 **The Global Commission on how to End the Diagnostic Odyssey for Children with a Rare Disease**, by Clifford Goldsmith, US Chief Medical Officer at Microsoft

14:10 – 14:25 **Innovative technologies and the future of diagnostics** by Helena Kääriäinen, Research Professor, National Institute of Health and Welfare, Helsinki Finland





14:25 – 15:10 **Panel debate: How to ensure early diagnosis in the Nordic countries?**
Moderator: Line Friis Frederiksen, biologist, science journalist
Panelists: Arvid Heiberg, Prof. (em) Department of Medical Genetics, Oslo University hospital, Norway, Allan Meldgaard Lund, Professor of pediatrics, Chief Physician, Center for Inherited Metabolic Diseases, Rigshospitalet, Denmark, Maria Montefusco, Chair, Rare Diseases Sweden and Hans Tómas Björnsson MD PhD, Associate prof., Faculty of Medicine, University of Iceland, Assistant prof. of pediatrics and genetics, Johns Hopkins School of Medicine, USA

15:10 – 15:30 BREAK

15:30 – 17:00 **THEME II: PATIENT EMPOWERMENT**

15:30 – 15:45 **The importance of empowerment for patients, relatives and society,** by Terkel Andersen, President of EURORDIS – Rare Diseases Europe

15:45 – 16:15 **3 x 10-minute perspectives on empowerment:**

- Micro/Patient level: Experiences of web-based adaptation training for people with rare diseases, by Sinikka Hiekkala, Project Manager, 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, Sweden

16:15 – 17:00 **Panel debate: How do we facilitate and improve empowerment nationally and cross country?**
Moderator: Line Friis Frederiksen, biologist, science journalist
Panelists: Terkel Andersen, President, EURORDIS; Andreas Habberstad, Project leader, The Norwegian Federation of Organisations of Disabled People (FFO), Guðrún Helga Harðardótti, Family Therapist and CEO, Einstök Börn, Iceland, member of SBONN and Stinus Lindgreen, MP, (the Danish Social-Liberal Party), Adnan Berberovic, Patient Representative, The Swedish Bleeding Disorder Society

17:00 **End of Day 1**

AGENDA - Day 2

NORDIC RARE DISEASE SUMMIT 2021

12:45 – 16:15 **TUESDAY 13 April 2021**

12:45 – 13:00 **Registration for virtual meeting**
Help Desk: +45 50 92 72 03 or +45 31 21 31 84

13:00 – 13:10 **RECAP of DAY 1**
Summary of key messages from Day 1 of the Nordic Rare Disease Summit: Theme I: Diagnostic Delay and Theme II: Patient Empowerment, by Moderator: Line Friis Frederiksen, biologist, science journalist

13:10 – 14:40 **THEME III: PATIENTS' ACCESS TO INNOVATION**

13:10 – 13:25 **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

13:25 – 13:40 **How the Nordic countries align with nine European ORPH-VAL principles for assessment of medicines for rare diseases,** by Lieven Annemans, Professor of health economics, Ghent University, Belgium, Member of ORPH-VAL Working Group

13:40 – 13:55 **Q&A session with** Yann Le Cam, Chief Executive Officer, EURORDIS - Rare Diseases Europe and Lieven Annemans, Professor of health economics, Ghent University, Belgium

13:55 – 14:40 **Panel debate: How can we improve patients' access to innovation?**
Moderator: Lieven Annemans, Professor of health economics, Ghent University, Belgium, Member of ORPH-VAL Working Group
Panelists: Yann Le Cam, CEO, EURORDIS, Ida Sofie Jensen, CEO, The Danish Association of the Pharmaceutical Industry (Lif), Lina Nordquist, Healthcare Spokesperson, MP, (The Liberal Party) Sweden and Giles Platford, President Europe and Canada Business Unit, Takeda





14:40 – 16:00 **COLLABORATING TO IMPROVE CONDITIONS FOR PEOPLE WITH RARE DISEASES**

14:40 – 14:55 **Nordic Roadmap for Rare Diseases** by Birthe Byskov Holm, Chair, Rare Diseases Denmark

14:55 – 15:10 **Nordic Summit Address,** From the first National Strategy to a Common Nordic Roadmap for Rare Diseases by Bertel Haarder, President of The Nordic Council, (MP) and former Minister of Health, Denmark

15:10 – 15:25 **BREAK**

15:25 – 16:00 **Reflections on Nordic Roadmap for Rare Diseases and priorities for the future** This session will gather key stakeholders from across the Nordic countries: Pasi Nevalainen, Head of Rare Diseases Unit, Senior consultant at Tampere University Hospital, Finland and Stinus Lindgreen, MP and Member of the Capital Regional Council (The Social-Liberal Party), Denmark, Nicklas Sandström (M), Vice Chairman Executive Board in Region Västerbotten, Sweden and Member of Delegation of Healthcare at SALAR and Arvid Heiberg, Prof. (em) Department of Medical genetics, Oslo University hospital, Norway.

Introductory reflections followed by interview by moderator Line Friis Frederiksen, biologist, science journalist

16:00 – 16:15 **Wrap-up** by Duarte Marchand, Head of Nordics, Takeda

16:15 **End of Nordic Rare Disease Summit 2021**

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 932 rare disease patient organizations in 73 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

Lægemiddelindustri foreningen

The following organizations have endorsed the Nordic Rare Disease Summit:

efpia European Federation of Pharmaceutical Industries and Associations

EUCOPE European Confederation of Pharmaceutical Entrepreneurs AISBL

Danmarks Bløderforening

IML Innovative Mindre Life Science Bolag

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), The Norwegian Association of the Pharmaceutical Industry's rules (LMI rules) 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 program will ahead of the event be notified to 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 Better Health for People, Brighter Future for the World. 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.