IDMC 12

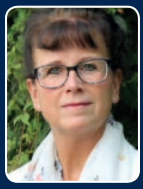
INTERNATIONAL
MYOTONIC
DYSTROPHY
CONSORTIUM
MEETING

JUNE 10–14, 2019
GOTHENBURG SWEDEN

WELCOME to GOTHENBURG!

For the first time, the IDMC meeting will be held in Sweden in the city of Gothenburg. We are looking forward to a scientifically interesting and important meeting with leading scientists in the field of Myotonic Dystrophy from all around the world. Together, we will present, share and discuss new scientific achievements. We welcome you all to Sweden and hope that we together will make an unforgettable IDMC-12 meeting.

Sincerely,
Local Organising Committee



Anne-Berit Ekström
idmc12

Christopher Lindberg
idmc12

Partners

MYOTONIC DYSTROPHY FOUNDATION

REGION VÄSTRA GÖTALAND
Habilitation & Health

MDA Muscular Dystrophy Association

AFMTÉLÉTHON
INNOVER POUR GUERIR

Myotonic Dystrophy

LUPIN NEUROSCIENCES

GIVE YOUR NDM PATIENTS

MOVEMENT WHEN IT MATTERS

NDM – Non-Dystrophic Myotonic disorders

NaMuscla:
1st licensed antimyotonic proven to:

- control the disabling symptoms of myotonia in adult NDM patients[1,2]
- improve patients' daily lives[1,2]
- provide generally well tolerated, long-term myotonia relief[3]

NaMuscla®
MEXILETINE

BREAK FREE FROM MYOTONIA

Abbreviated Prescribing Information: NaMuscla® (mexiletine)

Information about this product, including adverse reactions, precautions, contra-indications and method of use can be found at https://www.ema.europa.eu/en/medicines. Prescribers are recommended to consult the summary of product characteristics before prescribing. **Active Ingredients** Each capsule contains mexiletine hydrochloride corresponding to 166.62 mg mexiletine. **Indication** NaMuscla is indicated for the symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders. **Warnings & Precautions** Mexiletine may induce an arrhythmia or accentuate a pre-existing arrhythmia, either diagnosed or undiagnosed. **Information about this product, including adverse reactions, precautions, contra-indications and method of use can be found at www.lupin-neurosciences.com, https://www.ema.europa.eu/en/medicines Marketing Authorization Number** EU/1/18/1325/003. **Date of Preparation or Last Review** 23 Oct 2018 **Full prescribing information is available from** Lupin Europe GmbH. Hanauer Landstrasse 139–143, 60314 Frankfurt am Main, Germany **E-mail:** customerserviceLEG@lupin.com **Website(s):** www.lupin-neurosciences.com.

Marketing Authorization Address Lupin Europe GmbH Hanauer Landstrasse 139–143 60314 Frankfurt am Main Germany.

References:
1. Statland JM, *et al. JAMA* 2012;**308**(13):1357–65. **2.** Summary of Product Characteristics, NaMuscla® 167 mg hard capsules, Lupin Europe GmbH 2018. **3.** Suetterlin KJ, *et al. JAMA Neurology* 2015;**72**(12):1531–33.

Job Code: Lup_EMEA_Neuro_P_NA/24/07-05-2019
Date of Preparation: May 2019

PROGRAM OVERVIEW

MONDAY June 10
15:00 **Registration opens**
17:00-17:15 **Welcome and opening remarks:** Anne-Berit Ekström and Christopher Lindberg
17:15 - 17:45 **Introductory lectures:** Anders Olausson DM one of many rare disorders - experience of an international work in order to make these disorders known in the community
17:45 - 18:15 **Search for new neuromuscular disorders – The Gothenburg experience** Anders Oldfors
18:15 - 18:45 **A short history of Gothenburg** Ninna Boberg, Museum teacher, Göteborg City Museum
19:00- 21:00 **Welcome reception at Imagine** (Gothia Towers)

TUESDAY June 11
8:30-9:15 **Keyonote** Kaj Blennow Biomarkers in CSF in neurodegenerative disorders
09:15-10:00 **Cell Models for DM**
10:00-10:30 **Coffee break & visit to the exhibition**
10:30-12:00 **Animal Models and Tissue-specific Mechanisms**
12:00-13:00 **Lunch – Visit to posters & Exhibition** Open lunch meeting for TREAT-NMD Registry Organization and all interested. Room: R24
13:00-15:00 **Clinical Research**
15:00-15:30 **Coffee Break & Visit to Exhibition**
15:30-16:00 **Flash poster session**
16:00-17:30 **Break-out Session: Young Scientists** Room: G3
16:00 – 17:30 **Break-out Session: Classification of DM1** Room: R11+R12

WEDNESDAY
June 12

8:30-9:15
Keynote
David Nelson
Phenotype/genotype correlation in repeat expansion disorders

09:15-10:00
Pathogenic Mechanisms part I

10:00-10:30
Coffee break & visit to the exhibition

10:30-12:00
Pathogenic Mechanisms part II

12:00-13:00
LUNCH & Visit to the posters
Lunch session – How do we facilitate participation of unrepresented minorities in DM1 research. Room G2+G3
Poster view

13:00-13:30
Introductory speaker

13:30-15:00
Quality of Life and Disease Burden

15:00-15:30
Coffee break & Visit to Exhibition

15:30-16:00
Flash poster session

16:00-17:30
Break-out Session: Standards of Care
Room: G2+G3

THURSDAY
June 13

8:30-9:15
Keynote
Shoji Tsuji
Non-coding repeat expansion with same repeat motifs in three genes cause benign adult familial myoclonic epilepsy

09:15-10:00
Registries, diagnostic and genetic counselling

10:00-10:30
Coffee break & visit to the exhibition

10:30-12:00
Specific Disease Features

12:00-13:00
LUNCH & Visit to the posters

13:00-13:30
Introductory speaker

13:30-15:00
Drug development and delivery

15:00
Coffee break

15:30-16:30
Late Breaking news

16:15-17:15
Presentations from Patient organizations
Awards
Presentation of IDMC-13

19:00-23.00
Congress dinner at Universeum

FRIDAY
June 14

10:00-16:00
FAMILIES DAY at Dalheimers hus

KEYNOTE SPEAKERS

Biomarkers in CSF in neurodegenerative disorders

Kaj Blennow

Many neurodegenerative disorders such as Alzheimer's disease (AD), are notoriously difficult to diagnose on clinical grounds, especially in the early stages of disease. Further, the slow and variable clinical course of the disease makes it challenging to identify a drug effect on symptomatology evaluated by clinical rating scales. Thus, in trials on disease-modifying drug candidates, biomarkers are of importance, both to identify target engagement (pharmacodynamic markers) and to identify effects on neurodegeneration (downstream markers).

In AD, cerebrospinal fluid (CSF) biomarkers are increasingly used, and today the AD CSF biomarker toolbox includes total tau (T-tau) reflecting the intensity of neuronal degeneration, phosphorylated tau (P-tau) that correlates with brain tau pathology load, and ß-amyloid protein (Aß42 or Aß42/40 ratio) reflecting cortical Aß deposition.

These core CSF biomarkers have very consistently been found to have high diagnostic accuracy. Low CSF Aß42 show high concordance with amyloid PET measures of brain amyloidosis. High CSF T-tau adds to predict progression of symptoms, while high CSF P-tau adds specificity to differentiate from other disorders. The novel synaptic biomarker neurogranin is also seemingly specific for AD.

Recent additions to the AD CSF biomarker toolbox are the neurodegeneration biomarker neurofilament light (NFL) and the synaptic protein neurogranin. While CSF NFL tracks neurodegeneration in several brain disorders, increased CSF neurogranin is seemingly specific for AD, and predicts future rate of cognitive decline.

New ultra-sensitive analytical techniques allow for precise quantification of these biomarkers also in blood samples. Several studies using either immunoassay or mass spectrometry techniques show that low Aß42 (or Aß42/40) ratio in plasma correlates with amyloid deposition evaluated by PET scans. Plasma NFL levels correlate well with CSF levels, and show high promise as a tool to monitor neurodegeneration, not only in AD but also in many other neurodegenerative disorders. Methods to measure plasma levels of P-tau181 have recently been developed, showing a clear increase in AD. Although further studies are needed to validate findings, these blood biomarkers show promise for use as screening tools in the future, especially in the primary care setting, to rule out patients without biomarker evidence of neurodegeneration, which would limit costs for detailed evaluations at specialist clinics for a large proportion of patients.

CGG repeat expansion in FMR1: disorders, mechanisms and implications for human genetics.

David Nelson

Fragile X syndrome is a common cause of intellectual disability and autism. In nearly all cases it is caused by loss of function of FMR1 due to large expansions of a CGG repeat and subsequent down-regulation of transcription. Smaller expansions (premutations) that predispose to fragile X syndrome can also confer phenotypes, most prominently a late age of onset neurodegenerative disorders characterized by tremor, ataxia and cognitive decline (FXTAS). The mechanisms leading to these disorders are distinct, and multiple mechanisms may be involved in each. For example in FXTAS, evidence can be found for pathology stemming from both toxic gain of function of mRNAs (similar to myotonic dystrophy) and toxic small peptides produced by RAN translation. Lessons from dozens of human loci exhibiting similar repeat expansions suggest that this mechanism is common and could play a wider role in genetic contributions to human disorders and variation.

Non-coding repeat expansions with same repeat motifs in three genes cause benign adult familial myoclonic epilepsy.

Shoji Tsuji

Department of Molecular Neurology, Graduate School of Medicine, The University of Tokyo and Institute of Medical Genomics, International University of Health and Welfare

Unstable tandem repeat expansions have been shown to be involved in a wide variety of neurological diseases. Given a rapidly increasing number of diseases belonging to this group, it is expected that many more diseases await identification of causative genes. We have recently identified non-coding repeat expansions in benign adult familial myoclonic epilepsy (BAFME), an autosomal dominant disorder characterized by infrequent epilepsy and myoclonic tremor. The clue for identification of non-coding repeat expansion was obtained by an observation of a TTTTA pentanucleotide repeat located in intron 4 of SAMD12, which showed an apparently inconsistent transmission pattern in a family. An intensive search of the whole genome sequence data revealed TTTCA and TTTTA repeat expansions in intron 4 of SAMD12, which were found exclusively in the patients in the 51 families. RNA foci consisting of UUUCA repeats, but not of UUUUA repeats, were observed in neurons of the autopsied brains. Intriguingly, similar TTTCA and TTTTA repeat expansions were further identified in introns of TNRC6A and RAPGEF2, in patients with the clinical diagnosis with BAFME, who did not carry TTTCA repeat expansions in SAMD12. The findings that the same expanded repeat motifs in the three independent genes lead to BAFME phenotypes emphasize the role of TTTCA repeat expansions in BAFME, presumably through RNA-mediated toxicity, which was further supported by the presence of RNA foci without ubiquitinated inclusions. Based on these observations, we propose a new concept of “repeat motif-phenotype correlation”. We are currently applying this strategy to further identify non-coding repeat expansions in neurological diseases.

DETAILED PROGRAM

Monday 10 Jun 2019

15:00 - 17:00 **Registration opens**

Location: Conference foyer - G-hall

17:00 - 17:15 **Welcome and opening remarks: Anne-Berit Ekström and Christopher Lindberg**

Location: Main session hall - G2+G3

17:15 - 17:45 **Introductory lecture: Anders Olausson -Myotonic Dystrophy-One of many Rare Disorders- experience of an International work in order to make these disorders known in the community**

Location: Main session hall - G2+G3

17:45 - 18:15 **Introductory lecture: Anders Oldfors- Seach for new neuromuscular disorders – The Gothenburg experience**

Location: Main session hall - G2+G3

18:15 - 18:45 **History of Gothenburg - Ninna Boberg**

Location: Main session hall - G2+G3

Ninna Boberg, Museum teacher, Göteborg City Museum

19:00 - 21:00 **Welcome Reception hosted by the City of Gothenburg and Region Västra Götaland**

Location: Restaurant Imagine

The City of Gothenburg and Region Västra Götaland together with IDMC-12 has the pleasure to invite you to attend the Welcome Reception.

Mingle/finger food and a glass of wine or beer will be served. The Welcome Reception is included in the registration fee but registration is mandatory.

Tuesday 11 Jun 2019

08:00 - 08:45 **Registration**

Location: Conference foyer - G-hall

08:30 - 09:15 **Keynote - Kaj Blennow- Biomarkers in CSF in neurodegenerative disorders**

Location: Main session hall - G2+G3

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
08:30	Biomarkers in CSF in neurodegenerative disorders	Kaj Blennow	

09:15 - 10:00 **Oral - Cell models**

Location: Main session hall - G2+G3

Chairs: Laura RANUM, USA, Maurice SWANSON, USA

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
09:15	Premature Aging in DM1 Patient Derived Fibroblasts is Related to BMI-1 Pathway	Mikel García-Puga	S1-01
09:30	Excision of the CTG Repeat in DM1 Reverses DMPK Hypermethylation Depending on the Differentiation State of the Cell	Rachel Eiges	S1-02
09:45	Subnuclear Organisation in Myotonic Dystrophy Type I Cell Models	Monika Magon	S1-03

10:00 - 10:30 **Coffee break - Visit to posters & exhibition**

Location: Conference foyer - G-hall

10:30 - 12:00 **Oral - Animal Models and Tissue-specific Mechanisms**

Chairs: Christopher PEARSON, CANADA, Eric WANG, USA

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:30	Loss of Mbnl2 Gene in Glutamatergic Neurons Recapitulates DM1 Neuropsychopathology: Effects of the Atypical Antidepressant Mirtazapine	Carla Ramon-Duaso	S2-01

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:45	RNA Toxicity and Skeletal Muscle Regeneration	Mani S. Mahadevan	S2-02
11:00	Development of Mbnl Mutant and CUG Repeat-Expressing Stable Transgenic Zebrafish That Model Molecular and Physical Phenotypes of Myotonic Dystrophy	Melissa Hinman	S2-03
11:15	The Contribution of Nuclear and Cytoplasmic CELF1 Protein to Muscle Wasting in Myotonic Dystrophy Type 1	Diana Cox	S2-04
11:30	Heart-specific MBNL1/2 Double Knockout Mouse Model Recapitulates Dilated Cardiomyopathy and Arrhythmia-related Sudden Death in DM	Carol Seah	S2-05
11:45	Microsatellite Expansion Knockin Mouse Models for Myotonic Dystrophy Type 1	Curtis A. Nutter	S2-06

12:00 - 13:00 **Lunch, visit to posters & exhibition**

Location: Conference foyer - G-hall

12:00 - 13:00 **Open lunch meeting for TREAT-NMD Registry Organization and all interested**

Location: Room - R24

13:00 - 15:00 **Oral - Clinical Research: Methods Biomarkers Outcome Measures Trial Design etc**

Location: Main session hall - G2+G3
Charis: Chris TURNER, UK, Guillaume BASSEZ, France

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
13:00	Cognitive Behavioural Therapy in Patients with Severe Fatigue with Myotonic Dystrophy type 1: a Multicentre, Single-blind, Randomised Trial	Baziel van Engelen	S3-01

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
13:15	Genotype is Significantly Associated with Both Muscular and Cognitive Measures of Disease Phenotype in the OPTIMISTIC DM1 Patient Cohort	Sarah Cumming	S3-02
13:30	Muscle MRI in Myotonic Dystrophy Type 1 (DM1)	Matteo Garibaldi	S3-03
13:45	Longitudinal, Quantitative Assessment of Hand Muscle Strength Decay in Myotonic Dystrophy type 1 (DM1)	Erica Frezza	S3-04
14:00	Towards Validation of Specific Motor Outcome Measures in Myotonic Dystrophy Type 2	Emanuele Rastelli	S3-05
14:15	Exploratory Serum Biomarkers for Myotonic Dystrophy Type 1	Hanns Lochmuller	S3-06
14:30	PhenoDM1: The UK Myotonic Dystrophy Type 1 Multicenter Natural History Study	Nikoletta Nikolenko	S3-07
14:45	Contribution of Individual Muscle Groups to Functional Mobility Assessments in DM1	Katy Eichinger	S3-08

15:00 - 15:30 **Coffee break - Visit to posters & exhibition**

Location: Conference foyer - G-hall

15:30 - 16:00 **Flash poster session**

Location: Main session hall G2+G3
Chairs:
Anna-Karin KROKSMARK, Sweden
Stefan WINBLAD, Sweden

16:00 - 17:30 **Breakout session: Young Scientist**

Location: Room G2+G3
Chairs:
Frederica MONTAGNESE, Germany
Stephan WENNINGER, Germany
Peter MEINKE, Germany
Stefan HINTZE, Germany

16:00 - 17:30 **Classification of DM1**

Location: Room R11+12
Chair: Guillaume BASSEZ, France

Wednesday 12 Jun 2019

08:00 - 08:45 **Registration**

Location: Conference foyer - G-hall

08:30 - 09:15 **Keynote - David Nelson-CGG repeat expansion in fmr1: disorders, mechanisms and implications for human genetics**

Location: Main session hall - G2+G3

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
	CGG repeat expansion in FMR1: Disorders, mechanisms and implications for human genetics	David Nelson	

09:15 - 10:00 **Oral - Pathogenic mechanism part 1**

Location: Main session hall - G2+G3
Chairs: Nicolas SERGEANT, France, Tom COOPER, USA

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
09:15	Sense and Antisense RAN Proteins in Myotonic Dystrophy Type 1	John D.* Cleary	S4-01
09:30	Increased Muscleblind Levels by Chloroquine Treatment Improves Myotonic Dystrophy Type 1 Phenotypes in Vitro and in Vivo Models	Ariadna Bargiela	S4-02
09:45	Dissecting the Transcriptome of the DM1 CNS	Brittney A. Otero	S4-03

10:00 - 10:30 **Coffee break - Visit to posters & exhibition**

Location: Conference foyer - G-hall

10:30 - 12:00 **Oral - Pathogenic mechanism part 2**

Location: Main session hall - G2+G3
Chairs: Tetsuo ASHIZAWA, USA, Mar TULINIUS, Sweden

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:30	MSH3 Modifies Somatic Instability and Disease Severity in Myotonic Dystrophy Type 1 and Huntington Disease	Vilija Lomeikaite	S4-04

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:45	CRISPR-Mediated Expression of the Fetal Scn5a Isoform in Adult Mice Causes Conduction Defects and Arrhythmias	Paul Pang	S4-05
11:00	Congenital Myotonic Dystrophy Patients Exhibit Unique Splicing Dysregulation Compared to Adult-Onset DM1	Melissa Hale	S4-06
11:15	A CTG Repeat-Selective Chemical Screen Identifies Microtubule Inhibitors as Modulators of Toxic CUG RNA Levels	Kaalak Reddy	S4-07
11:30	miR-7 Restores Phenotypes in Myotonic Dystrophy Myoblasts by Repressing Hyperactivated Autophagy	Ruben Artero	S4-08
11:45	Signaling Pathways and RNA Toxicity	Mani S. Mahadevan	S4-09

12:00 - 13:00 **Lunch, visit to posters & exhibition**

Location: Conference foyer - G-hall

12:00 - 12:50 **Lunch session – How do we facilitate participation of unrepresented minorities in DM1 research**

Location: Room - G3
Chairs: Laura RANUM, USA, Erica SIMPSON, USA

13:00 - 13:30 **Report From the 4th Outcome Measures in Myotonic Dystrophy (OMMYD-4) Meeting**

Location: Main session hall - G2+G3
Chairs: Anna-Karin KROKSMARK, Sweden
Baziel van ENGELEN, The NETHERLANDS

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
	Report From the 4th Outcome Measures in Myotonic Dystrophy (OMMYD-4) Meeting	Cynthia Gagnon	

13:30 - 15:00

Oral - Quality of life and diseases burden

Location: Main session hall - G2+G3

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
13:30	Perceived Occupational Competence and Value of Everyday Activities, Fatigue and Quality of Life in Adults with Myotonic Dystrophy Type 1 (DM1)	Ulrika Edofsson	S5-01
13:45	An Interdisciplinary Therapy Clinic to Assess and Manage Swallowing, Secretions and Cough in Myotonic Dystrophy (DM1)	Jodi Allen	S5-02
14:00	Cough, Swallow Function and Neck Flexion Strength in Myotonic Dystrophy Type 1	Charlotte Massey	S5-03
14:15	Correlation Between Myotonic Dystrophy Health Index (MDHI) Scores and Clinical Function in Myotonic Dystrophy Clinical Research Network Participants	Chad Heatwole	S5-04
14:30	Performance of Activities of Daily Living in Congenital and Childhood Forms of Myotonic Dystrophy Type 1: A Population-based Study	Britt-Marie Eriksson	S5-05
14:45	Discrepancy Between Patient and Clinician Evaluation of Symptoms in Myotonic Dystrophy	Haruo Fujino	S5-06

15:00 - 15:30

Coffee break - Visit to posters & exhibition

Location: Conference foyer - G-hall

15:30 - 16:00

Flash poster session

Location: Poster area - Main Conference G2+G3
Chairs: Anna-Karin KROKSMARK, Sweden, Stefan WINBLAD, Sweden

16:00 - 17:30

Breakout sessions: Standards of Care

Location: Room G2+G3
Chairs Anne-Berit EKSTRÖM, Sweden, Molly WHITE, USA

Consensus-based Care Recommendations for Congenital and Childhood-onset Myotonic Dystrophy Type 1 (Nicholas Johnson)

Consensus-based Care Recommendations for Adults with Myotonic Dystrophy Type 1 (Tetsuo Ashizawa)

Consensus-based Care Recommendations for Adults with Myotonic Dystrophy Type 2 (Federica Montagnese)

Thursday 13 Jun 2019

08:00 - 08:45 **Registration**

Location: Conference foyer - G-hall

08:30 - 09:15 **Keynote - Shoji Tsuji- Non-coding repeat expansion with same repeat motifs in three genes cause benign adult familial myoclonic epilepsy**

Location: Main session hall - G2+G3

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
08:30	Non-coding repeat expansions with same repeat motifs in three genes cause benign adult familial myoclonic epilepsy	Shoji Tsuji	

09:15 - 10:00 **Oral - Registries, diagnostic and genetic councelling**

Location: Main session hall - G2+G3
Chairs: Adolfo Lopez de MUNAIN, Spain, Hanns LOCHMÜLLER, Cananda

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
09:15	The iDM-Scope Registry: An International Framework to Support Myotonic Dystrophy Translational Research	Guillaume Bassez	S6-01
09:30	Genetic Prevalence of Myotonic Dystrophy Types 1 And 2: A Population Cohort Study	Nicholas Johnson	S6-02
09:45	Being Diagnosed with Myotonic Dystrophy -- the Patients' Perspective	Miriam Rodrigues	S6-03

10:00 - 10:30 **Coffee break - Visit to posters & exhibition**

Location: Conference foyer - G-hall

10:30 - 12:00 **Oral - Specific disease features**

Location: Main session hall - G2+G3
Chairs: Giovanni MEOLA, Italy, Benedikt SCHOSER, Germany

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:30	A 9-year Longitudinal Brain Magnetic Resonance Imaging (MRI) Study in Myotonic Dystrophy Type 1 (DM1): Graph Theory Analysis of Disease Propagation	Garazi Labayru	S7-01

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
10:45	Sleep Disordered Breathing is Associated with Frontal and Parietal White Matter Loss in Myotonic Dystrophy Type 1	Mark Hamilton	S7-02
11:00	Diabetes, Metformin, and Cancer Risk in Myotonic Dystrophy Type I	Rotana Alsaggaf	S7-03
11:15	Cognitive Function in Congenital Myotonic Dystrophy: Cross-Sectional and Longitudinal Analyses	Melissa M. Dixon	S7-04
11:30	Tissue specific expression of expanded CUG repeat RNA reproduces cardiac features of myotonic dystrophy type 1	Ashish N. Rao	S7-05
11:45	Respiratory Management of Patients with Myotonic Dystrophy in Japan	Satoshi Kuru	S7-06

12:00 - 13:00 **Lunch, visit to posters & exhibition**

Location: Conference foyer - G-hall

13:00 - 13:30 **Introductory speaker Hanns Lochmuller Advances in drug development in DM**

Location: Main session hall - G2+G3
Chairs: Charles THORNTON, USA, Genevieve GOURDON, France

13:30 - 15:00 **Oral - Drug development and delivery**

Location: Main session hall - G2+G3

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
13:30	Correction of GSK3 Beta Increases Survival and Improves Growth, Neuromotor and Behavioral Activities of Mice With Congenital Myotonic Dystrophy	Lubov Timchenko	S8-01
13:45	Drug Combination Provides Additive and Synergistic Rescue of Mis-Splicing in Myotonic Dystrophy Type 1 Models	Jana Jenquin	S8-02
14:00	Systemic Delivery of Peptide-Conjugated Antisense Oligonucleotides Leads to Long-Lasting Correction of Myotonic Dystrophy Type I	Arnaud Klein	S8-03
14:15	Toward a Global Twin ASO Gene Therapy for DM1	Dominic Jauvin	S8-04

14:30	ARTHEx-DM, Antisense RNA Therapeutics in Myotonic Dystrophy	Maria Beatriz llamusi troisi	S8-05
14:45	A CRISPR-Cas13a Based Strategy That Tracks and Degrades Toxic RNA in Myotonic Dystrophy Type 1	Nan Zhang	S8-06

15:00 - 15:30 **Coffee break - Visit to posters & exhibition**

Location: Conference foyer - G-hall

15:30 - 16:15 **Late breaking news**

Location: Main session hall - G2+G3
Chairs: Tetsuo ASHIZAWA, USA, Maurice SWANSON, USA

Pres Time	Presentation title/Abstract title	Speakers/Authors	Pres
15:30	Targeted Delivery of Oligonucleotide Therapeutics to Muscle Reduces Toxic DMPK RNA	Mo Qatanani	S9-01
15:45	Muscleblind-like RNA binding proteins selectively interact with Kif1bα to localize Snap25 mRNA granules in neuronal processes which is impaired in an AAV based neuronal model of myotonic dystrophy DM1	Kathryn R. Moss	S9-02
16:00	Elevated levels of circular RNAs in myotonic dystrophy	Karol Czubak	S9-03

16:15 - 17:15 **Presentations from Patient organizations.**
Awards
Presentation of IDMC-13

Location: Main session hall - G2+G3

19:00 - 23:00 **Congress dinner**

Location: Universeum

Friday 14 Jun 2019

10:00 - 16:00 **FAMILIES DAY at Dalheimers hus**

Location: Dalheimers hus

For a detailed program, see page 27.

POSTERS

Posters - Cell Models for DM

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P1-001	Generation of Human 3D Skeletal Muscle Through Bioengineering for Preclinical Research in Myotonic Dystrophy	Juan M Fernandez-Costa
P1-002	Manipulating the Dose of Expanded DMPK Transcripts Using DCas9 in DM1 Myoblasts	Lise Ripken
P1-003	CTG Repeat Instability and DMPK Methylation in MSH2-deficient DM1 Patient Pluripotent Stem Cells	Silvie Franck
P1-004	Combination of Omics Approaches to Study Molecular Abnormalities in Individual Brain Cell Types of a DM1 Mouse Model	Anchel González-Barriga
P1-005	Development and Characterization of Pericyte-Derived IPSCs from DM1 Patients	Renée H. L. Raaijmakers
P1-006	Intrinsic Myogenic Potential of Skeletal Muscle-Derived Pericytes from Patients with Myotonic Dystrophy Type 1	C. Rosanne M. Ausems
P1-007	Elucidating CDK12 as Therapeutic Molecular Target in Myotonic Dystrophy	Anjani Kumari
P1-008	The Role of MBNL1 and 2 in Nuclear Foci Formation and Repeat Expansion Transcript Retention	Xiaomeng Xing
P1-009	Development of a Co-Culture System to Study the Interaction of Motoneurons and Skeletal Muscle Cells in Myotonic Dystrophy Type 1 (DM1)	Julie Tahraoui
P1-010	Use of Human Pluripotent Stem Cells to Study Myogenic and Neuronal Defects Associated with Myotonic Dystrophy Type 1	Antoine MERIEN
P1-011	The Role of MBNL1 and 2 in Nuclear Foci Formation and Repeat Expansion Transcript Retention	Xiaomeng Xing

Posters - Animal Models and Tissue-specific Mechanisms

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P2-001	Refinement of the DMSXL Mouse Phenotype	Aline Huguet
P2-002	CNS-associated Behavioral and Molecular Impairments in a Novel AAVCTG Based Neuronal Model of Myotonic Dystrophy Type I	Anwesha Banerjee
P2-003	Tissue Specific Expression Of Expanded CUG Repeat RNA Reproduces Cardiac Disease Features In An Inducible Mouse Model Of Myotonic Dystrophy Type 1	Ashish N. Rao

P2-004	Investigating the Contribution of Circadian Clock Disruption to DM1 Hypersomnolence	Belinda Pinto
P2-005	Elevated Exosomal Muscle-specific MiRNAs in Serum of DMSXL Mice and their Role in the Pathogenesis of the Disease	Chrystalla Mytidou
P2-006	Understanding Myotonic Dystrophy Through Transcriptomic Analysis of Humans, Mice, and Zebrafish	Jared Richardson
P2-007	Developing a BAC Transgenic Mouse Model of Myotonic Dystrophy Type 2 to Examine RNA and RAN Protein Effects	Kiruphagaran Thangaraju
P2-008	Development and Characterization of a Novel Microtubule-associated Tau Minigene Knockin Mouse Model Crossed with the DMSXL Mouse Model	Nicolas Sergeant
P2-009	Knockin Mice with Expanded CTG Repeats in Dmpk	Zhenzhi Tang

Posters - Clinical Research: Methods Biomarkers Outcome Measures Trial Design etc

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P3-001	Cerebrospinal Fluid Biomarkers in DM1: a 14-year Follow Up Study	Magnus Rudenholm
P3-002	Evaluation of Bioelectrical Impedance Analysis (BIA) as Potential Outcome Measure in Myotonic Dystrophy Type 1 (DM1): A Cross-sectional Study	Alessia Perna
P3-003	Regional Body Composition and Clinical Endpoints in Myotonic Dystrophy Type 2 (RACE-DM2)	Araya Puwanant
P3-004	Understanding Patient Preferences Role in The Medicinal Product Life Cycle: A Qualitative Study in Myotonic Dystrophy and Mitochondrial Disorders	Aura Cecilia Jimenez-Moreno
P3-005	Emotional Problems Among Individuals with Adult-Onset Myotonic Dystrophy Type 1 are Not Associated with Difficulties in Basic Emotional Processing	Claire Johnson
P3-006	Upper Limb Functions in Myotonic Dystrophy Type 1	Cynthia Gagnon
P3-007	First Report on a 10 Year Longitudinal Study of Balance and Muscle Force in the Myotonic Dystrophy Cohort in the Western Part of Sweden	Elisabet Hammarén
P3-008	Identification of Protein Markers Related to the Training-induced Positive Response Observed in Individuals with DM1	Elise Duchesne
P3-009	Lower-Limb Muscle Strength and Walking Capacity in Myotonic Dystrophy Type 1	Elise Duchesne
P3-010	Strength-Training as a Therapeutic Strategy in Myotonic Dystrophy Type 1	Elise Duchesne
P3-011	Patient Reported Outcome Measures in Myotonic Dystrophy Type 2	Federica Montagnese

P3-012	Age Related Cognitive Decline in Myotonic Dystrophy Type 1: An 11 Year Longitudinal Follow-up Study	Garazi Labayru
P3-013	Allele Length of the DMPK CTG Repeat is a Predictor of Progressive Myotonic Dystrophy Type 1 Phenotypes	Gayle Overend
P3-014	Longitudinal Multicenter Assessment of Cognition Using Computer Interface in Ambulatory Adults with Non-Congenital Myotonic Dystrophy Type 1 (DM1)	Gayle K Deutsch
P3-015	Outcome Measures Validity Testing: Appraisal in a Large International Cohort	Guillaume Bassez
P3-016	Cardiac Conduction Disorders as Markers of Cardiac Events but not Sudden Death in Myotonic Dystrophy Type 1	Hideki Itoh
P3-017	Detection of EEG Alterations in Myotonic Dystrophy Type I	Jacinda Sampson
P3-018	Gait Analysis Reveals Importance of Soleus Muscle in Ambulatory Myotonic Dystrophy Type 1	Jin-Sung Park
P3-019	Molecular Signatures in Blood Associated with Disease Severity in RNA-seq Profiles from DM1 Patients	Joanna Widomska
P3-020	Clinical Correlates of Chloride Channel 1 (CLCN1) Missplicing in Myotonic Dystrophy Type 1 (DM1)	Johanna Hamel
P3-021	Development of the Clinician-Completed Congenital Myotonic Dystrophy Type 1 Rating Scale (CDM1-RS)	Emily Fantelli
P3-022	A Longitudinal Analysis of Cognitive Flexibility and Value-Based Decision Making in Individuals with DM1	Kathleen Langbehn
P3-023	Cognitive Function in DM1 and Its Relationship to Brain Morphology	Kathleen Langbehn
P3-024	Central Nervous System Changes on MRI and Motor Function Correlations in People with Myotonic Dystrophy Type 1	Laurie Gutmann
P3-025	Dysarthria in the Childhood Forms of Myotonic Dystrophy type 1	Lotta Sjögreen
P3-026	Chewing Pattern and Chewing Efficiency in two Adults with the Classic form of Myotonic Dystrophy (DM1	Lotta Sjögreen
P3-027	Motor-free Measure of Intelligence for Adult with Myotonic Dystrophy Type 1 (DM1)	Louis Richer
P3-028	Quantified Muscle Testing for Maximal Knee Extensors Strength in Men with Myotonic Dystrophy Type 1: Intra-rater Reliability and Concomitant Validity	Marie-Pier Roussel
P3-029	Towards a Neurodevelopmental Approach to Social Cognition in Childhood DM1	Nathalie Angeard
P3-030	The Character of Pain in Patients with Myotonic Dystrophy Type 2	Olesja Parmová
P3-031	Volumetric Quantification of Calf Muscle Shape and Morphology from 3D MR Images: Fully Automated Deep LOGISMOS Approach	Peggy Nopoulos

P3-032	METMYD: Efficacy of Metformin on Mobility and Strength in Myotonic Dystrophy type 1 (DM1). Study Protocol Outline (Funded by AIFA)	Roberto Massa
P3-033	Bioelectric Impedance Analysis (BIA), Anthropometric and Nutritional Characteristics in Myotonic Dystrophy Type 2 (DM2) Patients	Erica Frezza
P3-034	Magnetic Resonance Imaging of Forearm Muscles in Myotonic Dystrophy Type 1 (DM 1): Functional Correlates	S H Subramony
P3-035	Positive and Negative Prediction of Respiratory Insufficiency in Myotonic Dystrophies	Stephan Wenninger
P3-036	Toward Validation of Extracellular mRNA Splicing Biomarkers of DM1 in Human Urine	Thurman Wheeler
P3-037	Assessment of Muscle Disease Progression in Myotonic Dystrophy Type 1 (DM1) by Longitudinal Muscle MRI Studies: Results of Our Pilot Experience	Tommaso Filippo Nicoletti

Posters - Pathogenic Mechanisms

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P4-001	Engineering Synthetic RNA Binding Proteins to Probe the Mechanisms of Myotonic Dystrophy and Development of Potential New Therapeutics	Carl Shotwell
P4-002	Aberrant Insulin Receptor Expression Is Associated with Insulin Resistance and Skeletal Muscle Atrophy in Myotonic Dystrophies	Rosanna Cardani
P4-003	RAN Translation in Myotonic Dystrophy Type 1: Does It Exist in Cell Cultures Derived from Patients' Tissues?	Emma Koehorst
P4-004	Muscle Single-Cell Analysis in Patients with Myotonic Dystrophy Type I	Judit Núñez-Manchón
P4-005	Mis-splicing of DMD Exon 78 in Heart Leads to Cardiac Contractile Dysfunction	Frédérique RAU
P4-006	3D-imaging of Single Myoblasts Derived From Myotonic Dystrophy Type I Patients': Studying the Relationship Between CTG Repeats, RNA-foci and MBNL1	Alfonsina Ballester-Lopez
P4-007	Measuring CTG Expansion in Myotonic Dystrophy Type I: Just Sizing the Tip of an Iceberg	Alfonsina Ballester-Lopez
P4-008	Resident Intron Misprocessing in GC-rich Microsatellite Expansion Diseases	Łukasz Sznajder
P4-009	Variant Repeats Protect Brain Structure and Function in DM1	Mark Hamilton
P4-010	Cancer Risk in Myotonic Dystrophy Type 1 is Linked to MiRNA200-141 Downregulation	Ander Matheu
P4-011	Muscle MRI Measures are Abnormal Early in Disease and Track Disease Progression: Support For Use as Biomarker in Clinical Trials	Laurie Gutmann

P4-012	Brain Imaging (MRI) Biomarkers of Disease Progression in DM1	Peg Nopoulos
P4-013	Comparison of Skeletal Muscle Transcriptome in Myotonic Dystrophy Type 1 and Type 2	Matthew Tanner
P4-014	PET Imaging of Tau Pathology in Myotonic Dystrophy Type 1: A Pilot Study	Caroline Dallaire-Théroux
P4-015	Abnormal Amygdala-striatal Network Relates to Apathy in Myotonic Dystrophy 1	Jacob Miller
P4-016	RBFOX Buffers MBNL Alternative Splicing Activity	Ryan Meng
P4-017	Knockout of Toxic DMPK Transcripts Using CRISPR/Cas9 Downregulates Inflammatory Cytokine Expression in a DM1 Lens Cell Line	Jeremy Rhodes
P4-018	Myotonic Dystrophy -- A Progeroid Disease?	Peter Meinke
P4-019	Resveratrol Corrects Aberrant Splicing of RYR1 and Ca2+ Signal in Myotonic Dystrophy Type 1 (DM1) Myotubes: Clues for a Therapeutic Trial	Massimo Santoro
P4-020	Nuclear Envelope Transmembrane Proteins in Myotonic Dystrophy Type 1 Muscle Cells	Stefan Hintze
P4-021	The Structure of DM1 Foci Analyzed by Super-Resolution Microscopy	Derick G. Wansink
P4-022	Antisense Transcription of the Unstable Triplet Repeat in DM1	Walther J.A.A. Van den Broek
P4-023	Elimination of the CUG Repeat from DMPK Transcripts Through a Natural Splice Mode	Remco T.P. van Cruchten
P4-024	DMPK MRNA Expression in Human Brain Throughout the Lifespan	Kathleen Langbehn
P4-025	Repeat Interruptions Modify Age at Onset in Myotonic Dystrophy Type 1 by Stabilizing DMPK Expansions in Somatic Cells	Jovan Pesovic
P4-026	The Role of Zinc Finger Protein 9, Encoded by a Myotonic Dystrophy Type 2 Gene, in Muscle Membrane Function	Lubov Timchenko

Posters - Quality of Life and Disease Burden

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P5-001	Eye Symptoms and Ophthalmologic Care in a Myotonic Dystrophy Type 1 (DM1) Population	Alain Geille
P5-002	Quality of Life, Cognitive and Behaviour Function in Children with Myotonic Dystrophy Type 1 in South-Eastern Norway	Anne-Britt Skarbø

P5-003	A Predictive Model to Assess Swallow and Cough Function in DM1	Charlotte Massey
P5-004	Measuring Activity and Sedentary Behaviour in Myotonic Dystrophy and Assessing the Use of Goal Setting to Increase Physical Activity	Charlotte Massey
P5-005	Gender Differences in Pain in Myotonic Dystrophy Type 1	Gro Solbakken
P5-006	Longitudinal Assessment of Orofacial Strength in Congenital Myotonic Dystrophy	Kiera Berggren
P5-007	Study of Care Practices for Patients with Myotonic Dystrophy in Japan -- Nationwide Patient Survey.	Masanori Takahashi
P5-008	Impact of Myotonic Dystrophy Type 1 on Activities of Daily Living	Nikoletta Nikolenko
P5-009	Association Between Two Measures of Activity Limitations in Myotonic Dystrophy Type 1.	Samar Muslemani
P5-010	Study of medical practices for patients with myotonic dystrophy in Japan -- Nationwide specialists survey	Tsuyoshi Matsumura
P5-011	Pilot Study of Cognitive-behavioral Therapy for Myotonic Dystrophy Type 1 Patients by Using a Biometric Information Monitor	Yukihiko Ueda
P5-012	The Ågrenska Family Program- Perceived usefulness and effects of family courses for rare diseases	AnnCatrin Röjvik
P5-013	Shared Space; A Creative Study of The Co-morbidity of Autism Spectrum Disorder and Myotonic Dystrophy in Young Adults and Their Parent(s)	Jacqueline Donachie

Posters - Clinical Guidelines Registries and Rehabilitation in DM

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P6-001	Oral Care Guidelines for Dental Teams Who See Patients with Myotonic Dystrophy Type 1 (DM1)	Åsa Mårtensson
P6-002	The UK Myotonic Dystrophy Patient Registry: An Essential Tool in the Facilitation of Translational Research	Chris Turner
P6-003	The Effects of a 12-week Strength-training Program on Fatigue, Sleepiness and Apathy in Men with Myotonic Dystrophy Type 1	Benjamin Gallais
P6-004	Combined Motor/Cognitive Training for Treatment of Balance Disorder in Myotonic Dystrophy Type 1 (DM1)	Elisabetta Bucci
P6-005	Registry of Myotonic Dystrophy in Japan: Current Status and Genotype-Phenotype Correlation.	Masanori Takahashi
P6-006	The Effect of a Behavioural Intervention on Skeletal Muscles in Myotonic Dystrophy Type 1 Assessed with Longitudinal Quantitative MRI	Linda Heskamp

P6-007	Affiliation to the Treat-NMD Consortium Using an Agreed Minimum Dataset Allows Small Registries and Large Registries to Collaborate Together	Richard Roxburgh
P6-008	How Can Occupational Therapists Address Sexuality with Patients Living with Myotonic Dystrophy?	Samar Muslemani

Posters - Diagnostic and Genetic Counselling

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P6-009	Molecular Diagnostics of Myotonic Dystrophy in the Era of Genome Scale DNA Sequencing	Jan Radvanszky
P6-010	Preimplantation Genetic Diagnosis for Female Carriers of Myotonic Dystrophy Type 1	Ute Hehr
P6-011	An Italian Experience of DM2 Genetic Testing	Annalisa Botta

Posters - Specific Disease Features—CNS Cardiac Gastro-Intestinal etc.

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P7-001	Structural Brain Imaging in Classical Myotonic Dystrophy Type 1 (DM1): A Voxel-Based Morphometry (VBM) and Diffusion Tensor Imaging (DTI) Study	Andone Sistiaga
P7-002	Structural Differences in Brain Magnetic Resonance Imaging in a Large Group of Polish Patients with Myotonic Dystrophy Type 1 and 2	Anna Łusakowska
P7-003	The Prevalence of Faecal Incontinence in Myotonic Dystrophy Type 1	Bob Ballantyne
P7-004	A Long-Term View of Cardiac Follow Up in Myotonic Dystrophy -- the Scottish Experience	Catherine McWilliam
P7-005	Incretin Secretion is Preserved in Myotonic Dystrophy Complicated with Diabetes Mellitus	Tsuyoshi Matsumura
P7-006	Cancer Frequency Among the Patients with Myotonic Dystrophy in Korea	Jin-Mo Park
P7-007	Oro-pharyngeal Dysphagia in Myotonic Dystrophy Type 1 (DM1): Identification of Sensory Changes Impacting Swallowing Function	Jodi Allen
P7-008	Comparison of Milestone Events in Myotonic Dystrophy Type 1 (DM1) and Type 2 (DM2)	Johanna Hamel
P7-009	Baseline Analyses of Brain Structural Features of DM1 and their Relationship with CTG Repeat Length	Mark Hamilton

P7-010	Review of Abnormalities of Calcium Metabolism, Calcium Signalling and Channelopathies in Myotonic Dystrophy	Martin Payne
P7-011	Differences in Splicing Defects Between Cortex and White Matter of Myotonic Dystrophy Type 1 Brain	Masamitsu Nishi
P7-012	Arrhythmic Risk Stratification and Risk Reduction in Patients with Myotonic Dystrophy	Matthew Wheeler
P7-013	Evaluation of Default Mode Functional Connectivity in Children with Congenital Myotonic Dystrophy	Melissa Dixon
P7-014	Heterozygous TNNT2 Variant in a DM1 Family With Cases of Sudden Cardiac Death	Rea Valaperta
P7-015	Trans-cortical and Spinal Reflex Plasticity in People with Myotonic Dystrophy Type I	Rich Shields
P7-016	Cholesteatoma in Early Onset Myotonic Dystrophy Type 1	Richard Petty
P7-017	Glucose Intolerance, Dyslipidemia and Liver Dysfunction in Myotonic Dystrophy	Riho Horie
P7-018	Cardiac Evaluation in Myotonic Dystrophy Type 1 (DM1)	Bob Ballantyne
P7-019	Cognitive Decline in DM1: a 14-year Longitudinal Study	Stefan Winblad
P7-020	Seven-year Follow-up Study of Fatigue in Patients with Myotonic Dystrophy Type 1	Vidosava Rakocevic Stojanovic
P7-021	Prevalence and Spectrum of Gastrointestinal Manifestations and Genotype-Phenotype Correlations in Myotonic Dystrophies (DM)	Vittorio Riso

Posters - Drug development and delivery

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P8-001	DMPK Promoter Silencing by CRISPRi as a New Therapeutic Strategy in Myotonic Dystrophy Type 1	Florent Porquet
P8-002	Falling Into Two Uracil: The Journey of U·U Recognizers	Raul Ondoño
P8-003	A Decoy-Based Gene Therapy to Inhibit RNA Toxicity Associated with Expanded CUG	Ludovic Arandel
P8-004	Safety and Tolerability of AMO-02 in a Phase 2 Clinical Trial for Congenital Myotonic Dystrophy Type 1	Hanns Lochmuller
P8-005	ARTHEx-DM, Antisense RNA Therapeutics in Myotonic Dystrophy	Maria Beatriz llamusi Troisi

P8-006	Slipped-CAG DNA Binding Small Molecule Induces Trinucleotide Repeat Contractions in Vivo; Part I	Stella Lanni
P8-007	Slipped-CAG DNA Binding Small Molecule Induces Trinucleotide Repeat Contractions in Vivo; Part II	Masayuki Nakamori
P8-008	Inducible and Specific CRISPR/Cas9-mediated Gene Editing in Cells Derived from Patients with Myotonic Dystrophy Type 1	Germana Falcone
P8-009	Progess in Therapeutic RNAi Gene Delivery for DM1	Joel Chamberlain

Posters - Miscellaneous

Location: Poster area - G4

Pres	Presentation title/Abstract title	Speakers/Authors
P9-001	Super Resolution Microscopy for Quantitative Analysis of Mutant DMPK Transcript Involved in DM1	Marzena Wojciechowska
P9-002	Rbfox1 Splicing Regulation Across a Broad Concentration Range	Joseph Ellis
P9-003	Bariatric Surgery in DM 1 -- a Long Term Follow-up	Karin Håkansson
P9-004	Factors Affecting Successive CTG/CAG Repeat Contractions in Rare DM1 Families	Shamima Islam Keka

PROGRAM IDMC-12 PATIENT- OCH ANHÖRIGDAG FREDAGEN 14 JUNI, DALHEIMERS HUS

9.00 – 10.00	**Registrering och kaffe**
10.00 – 11.00	**”Latest developments of gene modifying and other medical treatments in DM”** Senaste utvecklingen av genmodifierande och andra medicinska behandlingar vid DM.Professor Andy Berglund Department of Biological Science, University of Albany, USA (tolkas till svenska under presentationen)
11.00 -11.15	**Paus**
11.15 – 12.15	**”The latest achievements on clinical development in the field of DM”** De senaste kliniska landvinningarna inom DM. Professor Baziel van Engelen Department och Neurology, Nijmegen, Nederländerna (tolkas till svenska under presentationen)
12.15-13.15	**Lunch**
13.15 – 13.30	**Muskelsvindfonden Danmark ”Patientarbete i medlemsorganisationen”**
13.30 – 14.10	**Respirationsproblem som orsak till dagtrötthet** Holger Becker
	Munhälsa och orofaciala problem Lotta Sjögreen och Åsa Mårtensson
14.10 – 14.30	**Paus med kaffe**
14.30 – 15.45	**Motoriska funktionsnedsättningar** Anna-Karin Kroksmark, Marie Kierkegaard
	Kognition och beteende Anne-Berit Ekström, Stefan Winblad
	Vardagsaktivitet och aktivitetsförmåga Britt-Marie Eriksson, Ulrika Edofsson
15.45-16.00	**Frågor och Avslutning**

SOCIAL EVENTS.

WELCOME RECEPTION IN COLLABORATION WITH THE CITY OF GOTHENBURG AND REGION VÄSTRA GÖTALAND

– Monday June 10th at 19:00

The City of Gothenburg and Region Västra Götaland together with IDMC-12 has the pleasure to invite you to attend the Welcome Reception. The Reception will be held at Imagine Restaurant, located at the conference venue.

Mingle/finger food and a glass of wine or beer will be served. The Welcome Reception is included in the registration fee but registration is mandatory.

City of Gothenburg

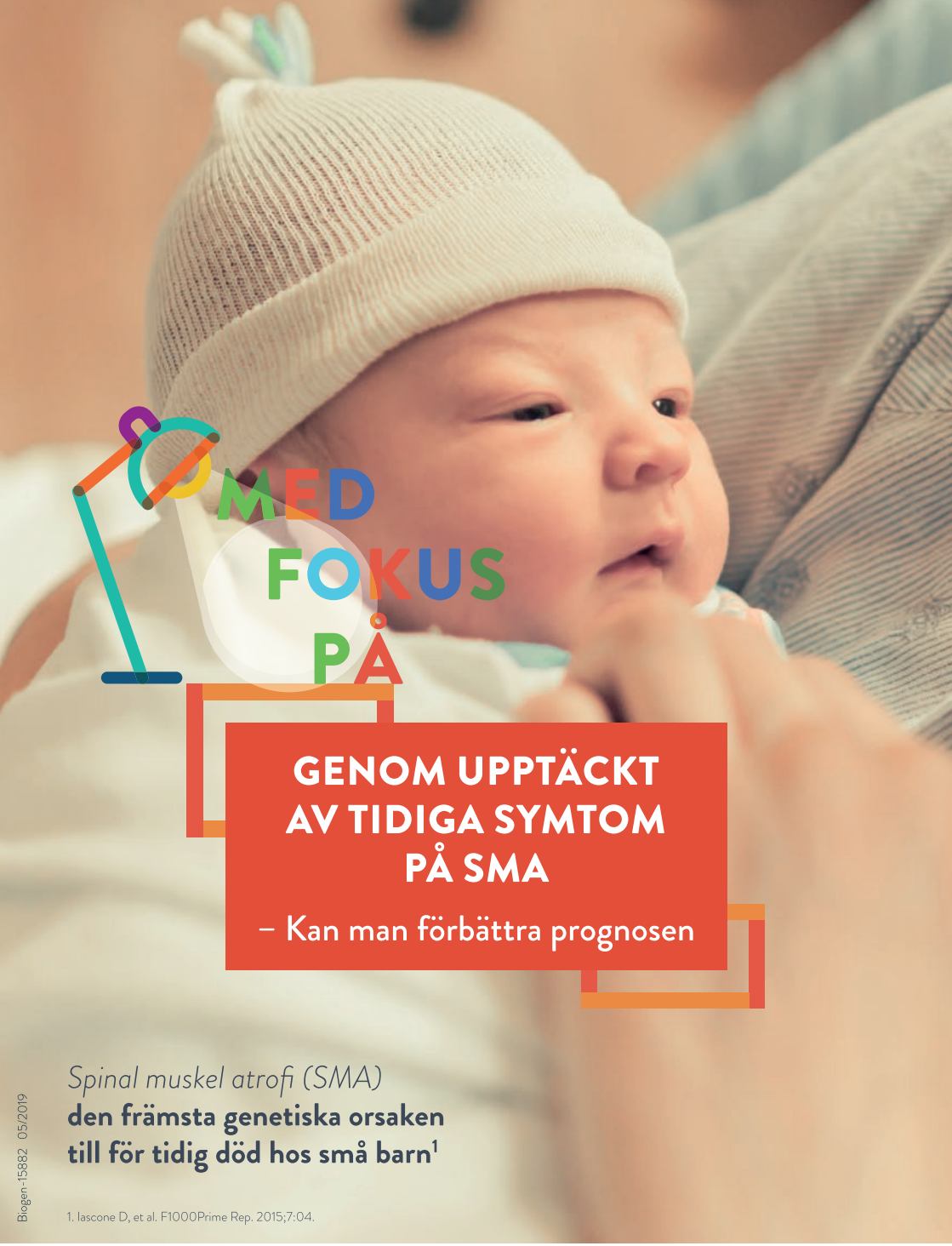
REGION VÄSTRA GÖTALAND

CONGRESS DINNER AT UNIVERSEUM

– Thursday June 13th at 19:00

The IDMC-12 Congress Dinner will take place at Universeum, Scandinavia's largest Science Centre which is located just nearby the conference venue. Imagine being on the bottom of the ocean and enjoy food and drink between two of Europe's largest aquariums in our Ocean Zone. Or admire the panoramic view of the magnificent Rainforest and all its colourful inhabitants.

Tickets for the conference dinner on Thursday evening can be bought via the online registration form. Dinner includes a 3 –course dinner, beverages, and entertainment. Price for conference delegates: SEK 300 ex VAT.



GENOM UPPTÄCKT AV TIDIGA SYMTOM PÅ SMA

– Kan man förbättra prognosen

Spinal muskel atrofi (SMA)
den främsta genetiska orsaken till för tidig död hos små barn[1]

1. Iascone D, et al. F1000Prime Rep. 2015;7:04.

Biogen-15882 05/2019

Biogen
Kanalvägen 10A | 194 61 Upplands Väsby
Tel 08-594 113 60

TOGETHER IN SMA™
Besök www.togetherinsma.se för mer kunskap om SMA

NOTES

GOLD SPONSORS

LUPIN NEUROSCIENCES

Biogen

BRONZE SPONSORS

Dyne THERAPEUTICS

SAREPTA THERAPEUTICS

EXHIBITOR

SANOFI GENZYME