

International Myotonic Dystrophy Consortium - 8
NOV 30 - DEC 3 | CLEARWATER BEACH FLORIDA



IDMC-8



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Welcome

Welcome to the 8th International Myotonic Dystrophy Consortium (IDMC-8) Meeting. The IDMC is an informal group of clinicians and research scientists who have a common interest in understanding the molecular events which result in the neuromuscular disease myotonic dystrophy (dystrophia myotonica, DM) and using this information to generate effective treatment strategies for this disease. The IDMC-8 meeting is not only designed to provide a forum for scientific presentations and discussions on the latest results in DM pathogenesis and therapy development but also to promote interactions between clinicians, basic scientists, patients and their families.

This is an exciting time in DM research. We hope you enjoy IDMC-8!

Tetsuo Ashizawa, M.D. and Maurice Swanson, Ph.D.
Co-Chairs, IDMC-8 Organizing Committee

Committees

Organizing Committee

Co-Chairs

Tetsuo Ashizawa, University of Florida, Gainesville, USA

Maurice Swanson, University of Florida, Gainesville, USA

Local Committee

Laura Ranum, University of Florida, Gainesville, USA

S.H. Subramony, University of Florida, Gainesville, USA

Basic Science Subcommittee

Geneviève Gourdon, Hôpital Necker-Enfants Malades, France

Shoichi Ishiura, Riken, Japan

Ralf Krahe, UTMDACC, USA

Darren Monckton, University of Glasgow, UK

Christopher Pearson, Hospital for Sick Children, Canada

Jack Puymirat, CHU Laval Research Center, Canada

Charles Thornton, University of Rochester, USA

Be Wieringa, University of Nijmegen, The Netherlands

Clinical Science Subcommittee

John Day, University of Minnesota, USA

Bruno Eymard, Hôpital Pitié-Salpêtrière, France

Tiemo Grimm, University of Würzburg, Germany

Giovanni Meola, University of Milan, Italy

Richard Moxley, University of Rochester, USA

Mark Rogers, University of Wales, England

Benedikt Schoser, University of Munich, Germany

Bjarne Udd, University of Helsinki and Tampere University Hospital, Finland

Conference Organizer

Catherine Gentilman, CMP, CGMP

*University of Florida, Department of Conferences
Gainesville, FL, USA*

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Congress General Information

Meeting Venue:

Sheraton Sand Key Resort
1160 Gulf Blvd.
Clearwater Beach, FL 33767-2799, USA
Tel: (727) 595-1611
<http://www.sheratonsandkey.com>

Registration:

The Registration desk is located outside of the meeting ballroom. The Registration desk will open Wednesday, November 30, 2011 at 15:00. The desk will remain open for the duration of the conference.

Oral Presentations:

Each speaker will have a 10 minute period for slide presentations with an additional five minutes for discussion. The program is packed and the session chairs will stick rigorously to the schedule. Speakers will have an opportunity to review their presentations in the speaker ready room.

Posters:

Posters should be attached on their boards on Thursday, December 1, 2011 before 10:00 and removed on Friday, December 2, 2011 by 18:00.

Meal Functions:

Breakfast, lunch, receptions and a closing dinner are included in the conference. Please refer to the agenda for the various locations.

Language:

The official language of the IDMC-8 is English.

Satellite Meetings:

Wednesday, November 30, 2011, 8:00 AM-3:00 PM

Outcome Measures for Myotonic Dystrophy

Organizer: Cynthia Gagnon

Sunday, December 4, 2011

Myotonic Dystrophy Foundation Patient Conference

Social Activities

Wednesday, November 30, 2011

Welcome Reception by the Gulf of Mexico

Sheraton Sand Key Resort Near the Turtle Bar

5:00 PM

Come join us for our welcome reception and enjoy the sunset.



Friday, December 2, 2011

Reception at the Salvador Dali Museum

Salvador Dali Museum

6:30-10:00 PM

Come join us for a wonderful reception at the Dali Museum. They have the world's most comprehensive collection of works by Salvador Dali. Transportation to and from the reception will be provided.

<http://www.salvadoralimuseum.org>

st. petersburg, florida



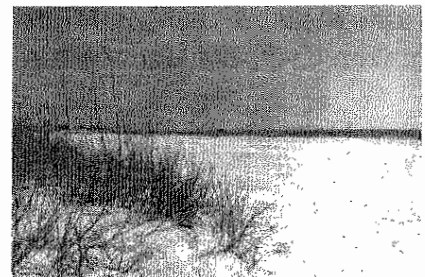
Saturday, December 3, 2011

Farewell Dinner on the Beach

Sheraton Sand Key Resort

6:30-10:00 PM

Come join us for our farewell dinner on the beach.



Sponsors

Association Française contre les Myopathies (AFM)

BioRad

University of Florida Center for NeuroGenetics

Genzyme

Isis Pharmaceuticals

Marigold Foundation

McKnight Brain Institute

Muscular Dystrophy Association (MDA)

Myotonic Dystrophy Foundation (MDF)

Myotonic Dystrophy Support Group (MDSG)

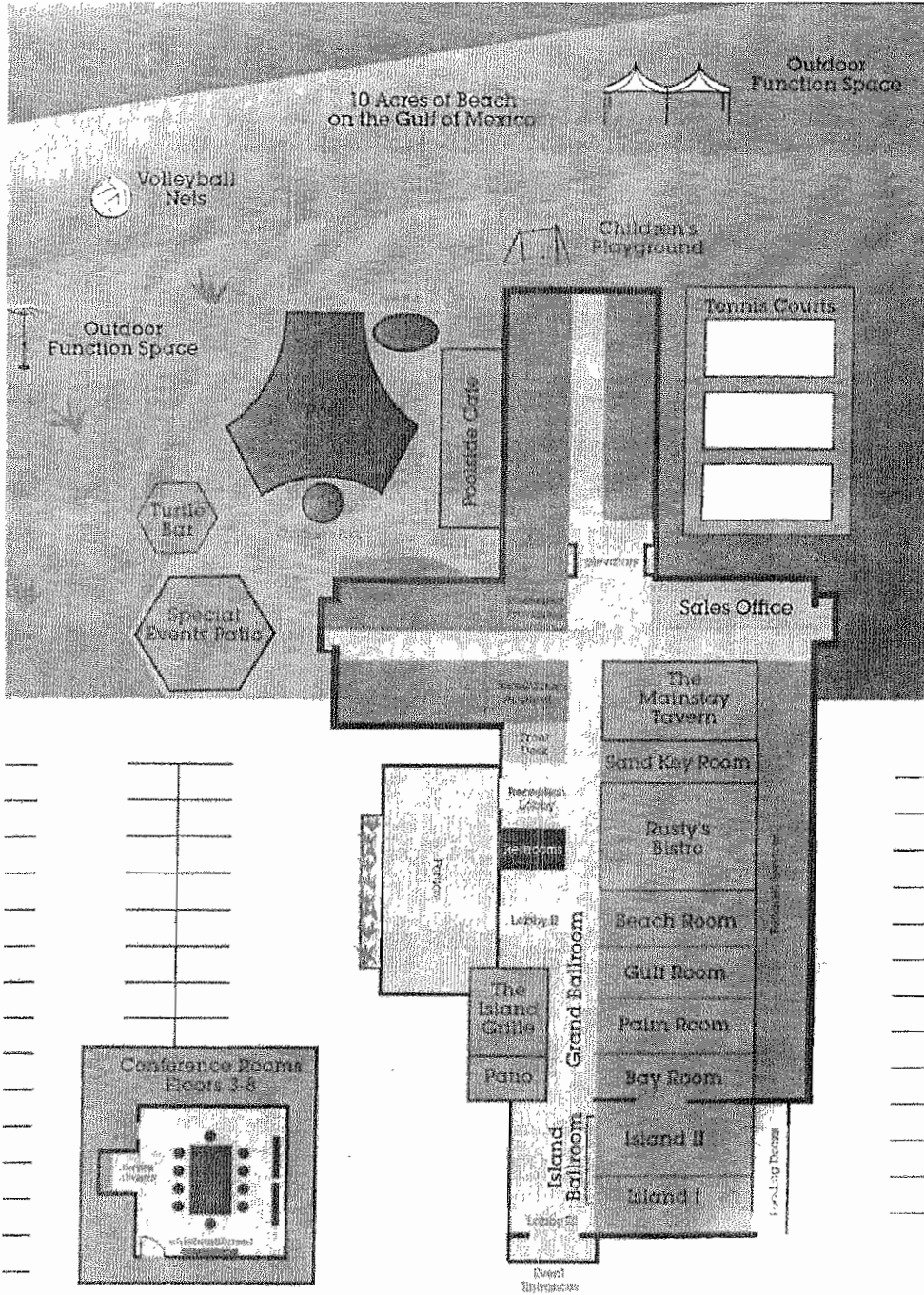
National Institutes of Health (NINDS, NIAMS, ORDR)

PTC Therapeutics

Shea-Robert Sheahan Trust

University of Florida Genetics Institute

Map of Hotel



IDMC-8 Scientific Program

Wednesday, November 30, 2011

3:00 PM/15:00	Registration and Check-In	Prefunction Area of Ballroom
5:00 PM/17:00	Welcome Reception	Sheraton Sand Key Resort (outside near the Turtle Bar)
7:00 - 9:30 PM	Session 1 - Opening Ceremony & Special Lectures	Island Ballroom
	S1.1 Welcome addresses and meeting information Tee Ashizawa and Maurice Swanson (Gainesville, USA)	
	Keynote Lectures <i>Chairs: Tee Ashizawa (Gainesville, USA)</i> <i>Thomas Cooper (Houston, USA)</i>	
7:30 - 8:15 PM	S1.2 Myotonic Dystrophy: The Disease Richard Moxley (Rochester, USA)	
8:30 - 9:15 PM	S1.3 The Transcriptome in Myotonic Dystrophy Christopher Burge (Boston, USA)	

Thursday, December 1, 2011

7:00	Registration/Information Desk Open	Prefunction Area of Ballroom
7:00-8:00	Breakfast	Grand Ballroom
8:00-12:00 PM	Session 2 - Disease Mechanisms I <i>Chairs: Ralf Krahe (Houston, USA)</i> <i>Shoichi Ishiura (Riken, Japan)</i>	Island Ballroom
8:00-8:30	S2.1 Overview Lecture: DNA Instability Meets RNA Toxicity Christopher Pearson (Toronto, Canada)	
Invited Talks from Abstracts (10 min talk, 5 min question period)		
8:30-8:45	S2.01 Intergenerational Transmission of the Myotonic Dystrophy Type 1 Mutation: Improved Risk Assessments for Congenital Disease F Morales, M Vásquez, P Cuenca, D Campos, C Santamaría, G del Valle, R Brian, M Sittenfeld, T Ashizawa & DG Monckton	
8:45-9:00	S2.02 Structure of Slipped-DNA Junctions Determine Correct or Mutagenic Repair of CAG/CTG Repeats MM. Slean, K Reddy, B Wu, KN Edamura, M Kekis, F Nelissen, R Aspers, M Tessari, OD. Schärer, SS. Wijmenga, CE. Pearson	
9:00-9:15	S2.03 MicroRNA Misregulated in DM1 Targets CUGBP1 E Querido, A Fournier, C Ménard, J Puymirat, P Chartrand	

IDMC-8 Scientific Program Continued

9:15-9:30	S2.04 Next Generation Sequencing Reveals miRNome Alterations in a DM1 <i>Drosophila</i> Model that are Conserved in Humans <u>JM Fernandez-Costa</u> , A Garcia-Lopez, A Felipo, A Bargiela, MB Llamusi, S Zuñiga, J Terol, V Fernandez, M Mata, M Perez-Alonso, AL deMunain and R Artero	
9:30-9:45	S2.05 The Processing of miR-1 is Altered in Myotonic Dystrophy <u>F Freyermuth</u> , F Rau, C Fugier, JP Villemin, MC Fischer, B Jost, D Dembele, G Gourdon, A Nicole, D Duboc, K Wahbi, JW Day, H Fujimura, MP Takahash, D Auboeuf, N Dreumont, D Furling, N Charlet-Berguerand	
9:45-10:00	S2.06 Correction of Processing and Decay of the Mutant DMPK and ZNF9 Transcripts in Myotonic Dystrophies Type 1 and Type 2 <u>K Jones</u> , B Jin, P Iakova, Bugiardini E, C Schneider-Gold, G Meola, NA Timchenko, LT Timchenko	
10:00-10:30	Coffee Break	Grand Ballroom
10:30-10:45	S2.07 CELF1-Mediated mRNA Decay Regulates Protein Secretion and Myogenesis and may be Impaired in Myotonic Dystrophy <u>AM Dickson</u> , CM López, JE Lee, T Nguyen, J Wilusz, CJ Wilusz	
10:45-11:00	S2.08 Evidence that Double Stranded RNA Activates the Innate Immune Response and Interferon Signalling in Myotonic Dystrophy <u>JD. Rhodes</u> , M Lott, SL. Russell, V Moulton, J Sanderson, M Wormstone, D Broadway	
11:00-11:15	S2.09 Mutant (CCUG)_{DM2} RNA Activates Cytoplasmic Stress Signaling Pathways in Myotonic Dystrophy Type 2 (DM2) Y Deng, KA. Baggerly, M Sirito, LL. Bachinski, B Schoser, B Udd, <u>R Krahe</u>	
11:15-11:30	S2.10 Fox Proteins Chase MBNL1 from CCUG Aggregates: An Explanation to the Lesser Severity of DM2? <u>C Sellier</u> , C Fugier, C Hammer, B Udd, J Puymirat, G Bassez, B Schoser, R Krahe, A Bergmann, D Furling, N Charlet-Berguerand	
11:30-11:45	S2.11 Misregulation of Cardiac MEF2 Gene Program in Myotonic Dystrophy <u>A Kalsotra</u> , R Singh, C Creighton, T Cooper	
11:45-12:00 PM	S2.12 Misregulated Alternative Splicing of CaV1.1 in Myotonic Dystrophy: Abnormal Calcium Channel Gating with Potential for Aggravation of Myopathy <u>ZZ Tang</u> , V Yarotsky, L Wei, K Sobczak, M Nakamori, K Eichinger, RT Moxley, RT Dirksen, CA Thornton	
12:00-12:15	S2.13 The RNA-binding protein Staufen1 controls myogenic differentiation <u>FX Laurent</u> , A Sureau, AF Klein, F Trouslard, D Furling, J Marie	

IDMC-8 Scientific Program Continued

12:00-2:00	<p>Lunch and Poster Viewing with Presenters</p> <p>Disease Mechanisms P1-P28 <i>Chairs: Thomas Cooper (Houston, USA)</i> <i>Sita Reddy (Los Angeles, USA)</i></p> <p>Cell and Animal Models Posters P29-P36 <i>Chairs: Denis Furling (Paris, France)</i> <i>Derick Wansink (Nijmegen, The Netherlands)</i></p>	Grand Ballroom
2:00-4:15	<p>Session 3 – Disease Mechanisms II <i>Chairs: David Brook (Nottingham, UK)</i> <i>Lubov Timchenko (Houston, USA)</i></p>	Island Ballroom
Invited Talks from Abstracts (10 min talk, 5 min question period)		
2:00-2:15	<p>S3.14 Repeat Associated Non-AUG (RAN) Translation: Implications for Myotonic Dystrophy <u>T Zu</u>, B Gibbens NS Doty, MS Swanson, LPW Ranum</p>	
2:15-2:30	<p>S3.15 Muscleblind Proteins Repress Aberrant Protein Expression Derived from Expanded Repeats <u>Y Kino</u>, C Washizu, M Kurowasa, Y Oma, S Ishiura, N Nukina</p>	
2:30-2:45	<p>S3.16 Non-AUG Initiated Translation Drives Inclusion Formation in FXTAS <u>PK Todd</u>, S Oh, A Krans, M Frazer, AJ Renoux, KM Scaglione, V Basur, K Elinitoba-Johnson, JP Vonsattel, ED. Louis, JP Taylor, HL Paulson</p>	
2:45-3:00	<p>S3.17 Elucidating the Role of NKX2-5 Expression in Skeletal Muscle <u>JT Gladman</u>, RS Yadava, M Manadl, Q Yu, MS Mahadevan</p>	
3:00-3:15	<p>S3.18 Highly Frequent Expansion and Contraction Mutations Drive Somatic DNA Diversity at the Myotonic Dystrophy Type 1 Locus <u>C F Higham</u>, F Morales, C A Cobbold, D T Haydon, D G Monckton</p>	
3:15-3:30	<p>S3.19 Detection of Slipped-DNAs at the Trinucleotide Repeats of the Myotonic Dystrophy Type I Disease Locus in Patient Tissues <u>MM Axford</u>, Y Wang, M Nakamori, M Zannis-Hadjopoulos, CA. Thornton, C E Pearson</p>	
3:30-3:45	<p>S3.20 Combinatorial Mutagenesis of the ZF RNA-binding Domains of MBNL1 Elucidate Distinct Classes of Pre-mRNA Regulatory Events <u>J Purcell</u>, JC Oddo, JA Berglund</p>	
3:45-4:00	<p>S3.21 The RNA-binding Protein Staufen1 is Increased in DM1 Skeletal Muscle and Regulates Pre-mRNA Splicing <u>A Ravel-Chapuis</u>, G Bélanger, RS Yadava, MS Mahadevan, L DesGroseillers, J Côté, BJ Jasmin</p>	
4:00-4:15	<p>S3.22 Transcriptome Regulation by Mbns via Splicing and Non-splicing Mechanisms <u>ET Wang</u>, S Jog, M Biancollela, TT Wang, D Treacy, GP Schroth, DE Housman, S Reddy, CB Burge</p>	

IDMC-8 Scientific Program Continued

4:15-4:45	Coffee Break	Grand Ballroom
4:45-7:15	Session 4 – Cell and Animal Models for DM <i>Chairs: Geneviève Gourdon (Paris, France)</i> <i>Be Wieringa (Nijmegen, Netherlands)</i>	Island Ballroom
4:45-5:15	S4.1 Overview Lecture: Models of DM Disease Mario Gomes-Pereira (Paris, France)	
5:15-5:30	S4.23 A Novel RNA-binding Protein Suppresses Expanded CUG-induced Muscle Wasting <u>M de Haro</u> , I Al-Ramahi, KR Jones, J Holth, L Timchenko, J Botas	
5:30-5:45	S4.24 Muscle Weakness and T-Tubule Disorganization Induced by DM1-like Splicing Defects of Bin1 in Mice <u>AF Klein</u> , C Fugier, C Hammer, A Ferry, L Garcia, G Butler-Browne, N Charlet-Berguerand, D Furling	
5:45-6:00	S4.25 Cell and Tissue in Vivo Distribution of Foci: Assessing a Myotonic Dystrophy Biomarker <u>C. Dogan</u> , L. Revillod , G Gourdon , D Furling , J Puymirat , R-K Gherardi , G Bassez	
6:00-6:15	S4.26 Reduction of CUGBP1 in a RNA Toxicity Mouse Model has Beneficial Effects YK Kim, M Mandal, <u>MS Mahadevan</u>	
6:15-6:30	S4.27 Development of a Muscleblind-like 1 (MBNL1) Overexpression Model to Assess the Use of MBNL1 as a Potential Therapeutic for Myotonic Dystrophy <u>CM Chamberlain</u> , G Chen, T Ebner, LPW Ranum	
6:30-6:45	S4.28 Mbnl3/Mbl1 Loss has a Synergistic Effect in the Development of DM1 Skeletal Muscle Disease S Tring, K Personius, L Comai, <u>S Reddy</u>	
6:45-7:00	S4.29 Muscleblind-like 2 Knockout Mice: A Model for Splicing Alterations and Neurological Changes in the DM Brain K Charizanis, <u>K-Y Lee</u> , MM ScottI, G Xia, L Shiue, MS Cline, M Ares Jr, T Kimura, MP Takahashi, H Fujimura, K Jinnai, H Yoshikawa, MS Swanson	
7:00-7:15	S4.30 Early Transcriptional Changes and Developmental Abnormalities in a Zebrafish Model of Myotonic Dystrophy Type 1 <u>PK Todd</u> , F Ackall, J Hur, HL Paulson, JJ. Dowling	
Dinner (at a venue of your choice) & Relaxation Dinner for Science Subcommittees and Session Chairs at Columbia Restaurant (http://www.columbiarestaurant.com)		

IDMC-8 Scientific Program Continued

Friday, December 2, 2011		
7:00-5:00 PM	Information Desk Open	Prefuction Area of Ballroom
7:00-8:00	Breakfast	Grand Ballroom
8:00-12:30 PM	Session 5 – Clinical Features and Biomarkers Discovery <i>Chairs: Peter Harper (Cardiff, UK)</i> <i>Bjarne Udd (Tampere, Finland)</i>	Island Ballroom
8:00-8:30	S5.1 Overview Lecture: DM: Challenges of a Multisystemic Disease Benedikt Schoser (Munich, Germany)	
Invited Talks from Abstracts (10 min talk, 5 min question period)		
8:30-8:45	S5.31 Congenital Myotonic Dystrophy: Canadian Surveillance and Cohort Study <i>C Campbell, R Taranik, S Venance, V Siu, S Levin, P Jacob</i>	
8:45-9:00	S5.32 A Randomized Controllef Trial of Prophylactic Implantable Carbioverter-Defibrillators is Feasible in the Myotonic Dystrophy Type 1 Population <i>WJ Groh, C Shen, PM Pascuzzi</i>	
9:00-9:15	S5.33 Defining Health Related Quality of Life in Congenital and Juvenile Myotonic Dystrophy: The First Step in the Development of a Disease-Specific Instrument <i>N Johnson; E Luebbe; E Eastwood; N Chin, RT Moxley, III, C Heatwole</i>	
9:15-9:30	S5.34 Improved Survival by Cardiac Electrophysiologic Study-based Management of Myotonic Dystrophy Type 1 Associated with Overt Disease of the Conduction System <i>D Duboc, C Meune, R Porcher, H Marc Bécane, A Lazarus, P Laforêt, T Stojkovic, A Béhin, H Radvanyi-Hoffmann, B Eymard, K Wahbi</i>	
9:30-9:45	S5.35 Disease Burden in DM1: Results from a 278 Patient Cross Sectional Study <i>C Heatwole, R Bode, N Johnson, C Quinn, W Martens, M McDermott, N Rothrock, C Thornton, B Vickrey, D Victorson, R Moxley</i>	
9:45-10:00	S5.36 Multicenter French-Quebec Myotonic Dystrophy Registry: A Platform for Research and Clinical Trials <i>G Bassez, JP Bouchard, C Guiraud-Dogan, J Mathieu, D Hamroun, L Cossette, C Bérourd, members of the Canadian and French DM Networks, Bruno Eymard, Jack Puymirat</i>	
10:00-10:30	Coffee Break	Grand Ballroom
10:30-10:45	S5.37 Development and Validation of a Disease Severity Index in DM1 <i>C Gagnon, J Mathieu</i>	
10:45-11:00	S5.38 Splicing Biomarkers of Disease Severity in Myotonic Dystrophy Type 1 <i>M Nakamori, K Sobczak, S Welle, A Puwanant, R Tawil, RJ Osborne, K Eichinger, RT Moxley III, CA Thornton</i>	

IDMC-8 Scientific Program Continued

11:00-11:15	S5.39 Gene Expression Biomarkers of Disease Severity in Myotonic Dystrophy Type 1 Muscle <u>KA Hagerman</u> , M Nakamori, S Welle, K Eichinger, J Dekdebrun, RT Moxley III, CA Thornton	
11:15-11:30	S5.40 Predictors of Tumor Development in Patients with Myotonic Dystrophy <u>M Das</u> , L Letren, JE Hilbert, WB Martens, RT Moxley III, MH Greene, SM Gadalla	
11:30-11:45	S5.41 Ophthalmologic Findings in Mexican Patients with Myotonic Dystrophy Type 1 <u>P Barojas</u> , JJ Magaña, G Ortega, N Leyva, B Cisneros, O Hernández-Hernández, E Barojas	
11:45-12:00 PM	S5.42 DM2 Linked Myopathy Caused by Uninterrupted Short (CCTG)⁵⁵ Repeat Expansion in ZNF9 Gene <u>T Suominen</u> , LL Bachinski, O Raheem, H Haapasalo, W Kress, R Krahe, B Udd	
12:00-12:15	S5.43 Evaluation of the Pathogenicity of Short Repeat Expansions at the DM2 Gene Locus <u>H Ellrott</u> , B Neitzel, A Abicht, B Czermin, C Doerner, I Holt, L Timchenko, W Kress, B Schoser	
12:15-12:30	S5.44 Cancer Risk in Patients with Myotonic Dystrophy <i>SM Gadalla, M Lund, RM Pfeiffer, S Gørtz, CM Mueller, RT Moxley III, SY Kristinsson, M Björkholm, FM Shebl, JE Hilbert, O Landgren, J Wohlfahrt, M Melbye, MH Greene</i>	
12:30-3:00	Lunch & Poster Viewing with Presenters Clinical Features and Biomarkers P37-P58 The Brain P59-P63 <i>Chairs: Tiemo Grimm (Würzburg, Germany)</i> <i>Baziel van Engelen (Nijmegen, The Netherlands)</i> Therapeutic Development P64-P81 <i>Chairs: Bruno Eymard (Paris, France)</i> <i>Mark Rogers (Wales, UK)</i>	Grand Ballroom
3:00-5:30	Session 6 – The Brain in Myotonic Dystrophy <i>Chairs: Bruno Eymard (Paris, France)</i> <i>Mark Rogers (Wales, UK)</i>	Island Ballroom
3:00-3:30	S6.1 Overview Lecture: DM and the Central Nervous System John Day (Palo Alto, USA)	
Invited Talks from Abstracts (10 min talk, 5 min question period)		

IDMC-8 Scientific Program Continued

3:30-3:45	S6.45 Widespread White Matter Microstructural Abnormalities in Congenital-onset and Juvenile-onset Myotonic Dystrophy <i>J Wozniak, B Mueller, C Bell, R Muetzel, K Lim, J Day</i>	
3:45-4:00	S6.46 Cognitive Decline in Myotonic Dystrophy Type 1 (DM1) <i>S Winblad, C Lindberg, L Samuelsson, G Meola</i>	
4:00-4:15	S6.47 Neuroradiologic Findings in a Series of 10 Patients with Childhood-onset Form of DM1 <i>A Jacquette, H Lemaitre, N Angeard, B Eymard, S Whalen, I Desguerre, F Brunelle, M Zilbovicius, D Heron, N Boddaert</i>	
4:15-4:30	S6.48 Neurocognitive Impairment in Childhood DM1: What Did We Learn, What Could We Expect? <i>N. Angeard, A. Jacquette, M. Gargiulo, B. Eymard, D. Héron</i>	
4:30-4:45	S6.49 Muscleblind-like 1 Knockout Mice Reveal Novel Splicing Defects in the Myotonic Dystrophy Brain <i>T Kimura, K-Y Lee, K Suenaga, M Nakamori, Y Tatsumi, MP Takahashi, H Fujimura, K Jinnai, H Yoshikawa, H Du, M Ares, Jr., MS Swanson</i>	
4:45-5:00	S6.50 Expression and Post-Translational Modification of CELF Family Proteins in DM1 Brains with a Differential Mis-splicing of Tau Exon 10 <i>CM Dhaenens, ML Frandemiche, FJ Fernandez-Gomez, C Carpentier, H Tran, A Sistiaga, M Goicoechea, H Obriot, S Eddarkaoui, MH Gavaert, B Sablonnière, L Buée, S Schraen-Maschke, A Lopez de Munain, N Sergeant, ML Caillet-Boudin</i>	
6:00	Shuttle leaves from the Sheraton to the Dali	
6:30-10:00	Science and Art: Trip to the Salvador Dali Museum Visit and reception at the new Salvador Dali museum in St Petersburg	Dali Museum
Saturday, December 3, 2011		
7:00-6:00 PM	Information Desk Open	Prefuction Area of Ballroom
7:00-8:00	Breakfast	Grand Ballroom
8:00-12:30 PM	Session 7 – Therapeutic Development <i>Chairs: Jack Puymirat (Quebec, Canada) Charles Thornton (Rochester, USA)</i>	Island Ballroom
Invited Talks from Abstracts (10 min talk, 5 min question period)		
8:00-8:15	S7.51 Antisense Approaches for Myotonic Dystrophy <i>J Puymirat, G Doucet, G Gourdon, D Furling, G Bassez and all members of the DM French-Canadian Network</i>	

IDMC-8 Scientific Program Continued

8:15-8:30	S7.52 Body-wide Reversal of Myotonia and Rescue of Myopathy in a Transgenic Mouse Model of DM1 by RNase H-Active Antisense Oligonucleotides (ASOs) <u>TM Wheeler</u> , AJ Leger, SK Pandey, AR MacLeod, M Nakamori, SH Cheng, BM Wentworth, CF Bennett, CA Thornton	
8:30-8:45	S7.53 RNase H-Mediated Degradation of Toxic RNA in Myotonic Dystrophy Type 1 <u>JE Lee</u> , TA Cooper	
8:45-9:00	S7.54 Enhanced Sensitivity of Nuclear-retained RNA Transcripts for RNAase H Active Antisense Oligonucleotides (ASOs): An application for Myotonic Dystrophy Type 1 (DM1) Therapy <u>SK Pandey</u> , TM Wheeler, AJ Leger, SH Cheng, BM Wentworth, CA Thornton, CF Bennett, Art Macleod	
9:00-9:15	S7.55 Potential Therapeutic Agents for Treatment of Myotonic Dystrophy; Synthesis and Evaluation of Pentamidine Analogs <u>MJ Bodner</u> , L Coonrod, D Farnsworth, MM Haley, JA Berglund	
9:15-9:30	S7.56 Systemic Delivery of RNAi Hairpins as a Treatment for DM1 in the HSALR Transgenic Mouse E Stepniak-Konieczna, CA Stoltzman, J Wei, DR Bisset, E Finn, JM Allen, CA Thornton, <u>JR Chamberlain</u>	
9:30-9:45	S7.57 In Vivo Discovery of a Peptide that Prevents CUG RNA Hairpin Formation and Reverses RNA Toxicity in Myotonic Dystrophy Models A García-López, B Llamusi, M Orzáez, E Pérez-Payá, RD Artero, <u>A. López Castel</u>	
9:45-10:00	S7.58 Silencing of CUG Expansion RNA in Myotonic Dystrophy 1 Mouse Models Using Adeno-associated Virus <u>Z Gao</u> , KR Clark, TA Cooper	
10:00-10:30	Coffee Break	Grand Ballroom
10:30-10:45	S7.59 Truncated Muscleblind-like Factor 1 Rescue Several Mis-splicing Events in Myotonic Dystrophy H Tran, C Carpentier, CM Dhaenens, FJ Fernandez-Gomez, C Lemercier, S Eddarkaoui, R Caillierez, S Bégard, H Obriot, N Charlet-Berguerand, S Schraen-Maschke, N Deglon, M Colin, L Buée, D Furling, ML Caillet-Boudin, <u>N Sergeant</u>	
10:45-11:00	S7.60 Splicing of CUG Repeats in the DMPK 3' UTR Attenuates DM1 Pathology ET Wang, <u>TT Wang</u> , D Treacy, C Burge, D Housman	
11:00-11:15	S7.61 Systemic Delivery of Conjugated (CAG)_n Oligonucleotides to Treat DM1 <u>S Mulders</u> , W van den Broek, A González-Barriga, B Aguilera, J van de Giessen, R Verheul, B Wieringa, J van Deutekom, DG Wansink	
11:15-11:30	S7.62 Some Observations on Oligo-based Therapy for Myotonic Dystrophy <u>JD Brook</u> , TE Robinson, A, Ketley, X Li, S Arya, I Udosen, J Granados Riveron, D Furling, CJ Hayes	

IDMC-8 Scientific Program Continued

11:30-11:45	S7.63 Mechanistic Studies on the Use of Triplet Repeat Antisense Oligonucleotides in Myotonic Dystrophy <i>A González-Barriga, S Mulders, A Gudde, I van Kessel, W van den Broek, J van de Giessen, J van Deutekom, B Wieringa, DG Wansink</i>	
11:45-12:00	S7.64 Stabilization of Expanded (CTG)•(CAG) Repeats by Antisense Oligonucleotides <i>M Nakamori, G Gourdon, CA Thornton</i>	
12:00-2:00	Lunch	Grand Ballroom
2:00-6:00	Session 8 – Interactive Session <i>Chairs: Valerie Cwik (MDA)</i> <i>Darren Monckton (Glasgow, UK)</i>	
2:00-2:30	S8.1 Disease Mechanism Highlights <i>Mani Mahadevan (Charlottesville, USA)</i>	
2:30-3:00	S8.2 Clinical Science Highlights <i>Tee Ashizawa (Gainesville, USA)</i>	
3:00-3:30	S8.3 Therapy Highlights <i>Andrew Berglund (Eugene, USA)</i>	
3:30-4:30	S8.4 Questions from Patients and Families	
4:30-5:45	S8.5 Advocacy Group Presentations	
5:45-6:15	S8.6 IDMC and Steinert Award Presentations	
6:30-10:00	Farewell Gala Dinner on the Beach	Sheraton Sand Key Resort Beach

ABSTRACTS and POSTER DESCRIPTIONS

The Abstracts and Poster descriptions (pages 18 through 164)
have been removed from this document to honor confidentiality requirements

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