“Lipodystrophy in 2014: Leptin and Beyond”
Scientific Program
University of Michigan, Ann Arbor

Day One, Daytime Program: October 17, 2014
North Campus Research Complex
2800 Plymouth Road, Ann Arbor, MI 48105

12:00 pm – 1:30 pm  Registration and Networking

1:30 pm – 1:45 pm  Welcome
Research Auditorium
Steve Groft, NCATS
Chuck Burant, MMOC

1:45 pm – 2:20 pm  Overview of Lipodystrophy Syndromes
Research Auditorium
Abhimanyu Garg, UTSW

2:20 pm – 4:30 pm  Session One: Pathways in Disease Development
Research Auditorium
Chair: Alan Saltiel, Life Sciences Institute

2:20-3:00  Pathways from Lipodystrophy to Clinical Disease
Robert Semple, Cambridge, UK

3:00-3:30 The unknown world of seipin
Jocelyn Magre, Universite de Nantes, France

3:30-4:00 Organising adipogenesis: Seipin as a molecular scaffold
Justin Rochford, Abardeen, UK

4:00-4:30 Nuclear lamins in health and disease
Stephen Young, UCLA

4:30 pm-4:45 pm  Break

4:45 pm-5:45 pm  Late-Breaking Key Note Lecture
New genes and pathways in regulation of adipogenesis, obesity and metabolic syndrome
Arya Mani, Yale University

5:45 pm – 6: 45 pm  Poster Session and Welcome Reception
Dining Hall

6:45 pm – 7:00 pm  Shuttles from NCRC to Sheraton Ann Arbor

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Day One, Evening Program: October 17, 2014
Sheraton Ann Arbor
3200 Boardwalk, Ann Arbor, MI 48108

7:30 pm – 9:30 pm  Dinner Symposium
Grande Ballroom
Chair: TBD

Panel Discussion: The Advantages and Challenges of Forming a Clinical Research Network for Rare Diseases: Are We Up for It?

What does the network bring to the table?
Steven Groft, NCATS

European network and perspective
David Araujo, Spain

Lipodystrophy Connect
Debbie Jae, PatientCrossroads

The charge
Elif A. Oral, UM
# “Lipodystrophy in 2014: Leptin and Beyond”

## Scientific Program

*University of Michigan, Ann Arbor*

### Day Two, Daytime Program: October 18, 2014

**North Campus Research Complex**  
2800 Plymouth Road, Ann Arbor, MI 48105

<table>
<thead>
<tr>
<th>Time</th>
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<tbody>
<tr>
<td><strong>7:00 am – 8:00 am</strong></td>
<td>Registration and Networking</td>
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| **8:00 am – 10:00 am** | **Session One: Adipogenesis and Adipocytokines (Not Leptin)**  
*Research Auditorium*  
Chair: Carol Haft, NIDDK  
**8:00-8:30**  
*Transcriptional networks regulating browning of human adipocytes*  
Susanne Mandrup, University of Southern Denmark, Odense, Denmark  
**8:30-9:00**  
*Bone marrow adiposity and lipodystrophy*  
Cliff Rosen, Tufts University Medical Center  
**9:00-9:30**  
*Marrow adipose tissue: Metabolism and endocrine functions*  
Ormond MacDougald, UM  
**9:30-10:00**  
*A subcutaneous adipose tissue-liver axis in the control of hepatic gluconeogenesis*  
Shannon Reilly, UM  |
| **10:00 am – 10:15 am** | Break  
*Atrium* |
| **10:15 am – 11:40 am** | **Focus Lectures**  
*Research Auditorium*  
Chair: Carol Haft, NIDDK  
**10:15-11:00**  
*Inventing new medicines. FGF21 story*  
Alexei Kharitonenkov, Indiana University (Bloomington, IN)  
**11:00-11:40**  
*Pathophysiological aspects of genetic and acquired lipodystrophy syndromes*  
Jacqueline Capeau, UPMC INSERM, Paris, France  |
| **11:40 am – 12:30 pm** | Lunch  
*Dining Hall* |

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12:30 pm – 2:00 pm  
Session Two: Models of Disease
Research Auditorium
Chair: TBD

12:30-1:00  
Phenotyping the Agpat2 knock-out lipodystrophic mice: some new observations  
Anil Agarwal, UTSW

1:00-1:30  
Mechanisms underlying sex differences in adiposity  
Karen Reue, UCLA

1:30-2:00  
Genome editing in pluripotent cells to generate human models of lipodystrophy  
Chad Cowan, Harvard

2:00 pm – 4:00 pm  
Session Three: Clinical Challenges
Research Auditorium
Chair: Abhimanyu Garg, UTSW

2:00-2:30  
Reproductive challenges: a great model for PCO-S  
Andrea Dunaif, Northwestern University

2:30-3:00  
Non-alcoholic steatohepatitis: Is there a benefit to study the rare?  
Arun Sanyal, Virginia Commonwealth University

3:00-3:30  
Multisystemic lipodystrophy syndromes: when there is more than lipodystrophy  
David Araujo, Universidad de Santiago, Spain

3:30-4:00  
Short talks

3:30-3:45  
Prevalence and mechanisms of polycystic ovarian syndrome in familial partial lipodystrophy, Dunnigan variety  
Chandna Vasani et. al, UTSW

3:45-4:00  
Hypothalamic tumors mimicking acquired generalized lipodystrophy  
Nivedita Patni et.al, UTSW

4:00 pm – 4:15 pm  
Break
Atrium

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<tr>
<td>4:15 pm – 5:45 pm</td>
<td>Session Four: Treatment of Lipodystrophy Syndromes (All about Leptin)</td>
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<td>Research Auditorium</td>
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<td>Chair: Phillip Gorden, NIDDK</td>
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<td>4:15-4:55</td>
<td>Overview of lipodystrophy treatment with metroleptin</td>
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<td>Rebecca Brown, NIDDK</td>
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<td>4:55-5:15</td>
<td>Leptin therapy in lipodystrophy: UT Southwestern experience</td>
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<td>Vinaya Simha, Mayo Clinic</td>
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<td>5:15-5:45</td>
<td>Translational research of leptin for lipodystrophy in Japan</td>
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<td>Ken Ebihara, Kyoto, Japan</td>
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<td>5:45 pm – 6:45 pm</td>
<td>Poster Session</td>
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<td>Dining Hall</td>
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Sheraton Ann Arbor
3200 Boardwalk, Ann Arbor, MI 48108

7:30 pm – 9:30 pm  Dinner Symposium
Grande Ballroom
Chair: TBD

Panel Discussion: All about Patients

A unique personal perspective on FPLD
Samantha Pulliam, Harvard

Discussion on patient priorities
Elaine Cochran, NIDDK

How to grow a voice for Lipodystrophy United
Andra Stratton, Lipodystrophy United

Open patient platform
### “Lipodystrophy in 2014: Leptin and Beyond”
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**Day Three, Daytime Program: October 19, 2014**

North Campus Research Complex  
2800 Plymouth Road, Ann Arbor, MI 48105

<table>
<thead>
<tr>
<th>Time</th>
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<td>7:00 am – 8:00 am</td>
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| 7:30 am – 7:40 am | Welcome and Goals for the Day  
*Research Auditorium*  
Simeon Taylor, University of Maryland                                                   |
| 7:40 am – 8:40 am | **Session One: Some more on Leptin**  
*Research Auditorium*  
Chair: Simeon Taylor, University of Maryland                                             |
| 7:40-8:10     | Novel molecules on the leptin pathway  
Martin Myers, UM                                                                         |
| 8:10-8:40     | **Short talks**  
8:10-8:25 One year metreleptin improves insulin secretion in patients with lipodystrophy syndromes  
Camille Valtier et.al, INSERM, Paris, France                                               |
|               | 8:25-8:40 Lessons from treatment of children with congenital leptin deficiency with metreleptin  
Martin Wabitsch, Division of Pediatric Endocrinology and Diabetes, University Medical Center, Ulm, Germany |
| 8:40 am – 11:05 pm | **Session Two: Unmet Medical Need Beyond Leptin**  
*Research Auditorium*  
Chair: Simeon Taylor, University of Maryland                                              |
| 8:40-9:10     | NASH and obethicolic acid  
Abhimanyu Garg, UTSW                                                                      |
| 9:10-9:40     | Antisense drugs as novel insulin sensitizers: bench to clinic  
Sanjay Bhanot, ISIS Pharmaceuticals                                                          |
| 9:40-10:10    | Novel treatments for hyperlipidemia  
Andres Digenio, ISIS Pharmaceuticals                                                           |
| 10:10-10:40   | Designer combination hormonal therapies  
Matthias Tschöp, IDO, Munich                                                               |

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10:40 am – 10:55 am     Break

10:55-11:25-11:10     Short talks
  10:55—11:10 Forced PPARγ1 expression confers adipogenic competence to BSCL type 1 and type 2 human dermal fibroblasts.
  Rosa M. Moro et. al. CIEMAT Centre for biomedical research on rare diseases, Madrid, Spain

  11:10-11:25 The brown fat enriched secreted factor Nrg-4 preserves metabolic homeostasis through attenuating hepatic lipogenesis
  Guo-Xiao Wang, LSI, UM

11:25 am – 11:55 am     Focus Lecture
Lessons from an accomplished warrior: NEPTUNE as a case study for ORD CRN
Matthias Kretzler, UM

11:55 am – 12:15 pm     Agenda for the Year & Concluding Remarks
Phillip Gorden, NIDDK