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# GRAND CHALLENGES IN PARKINSON'S DISEASE

*GBA1 and LRRK2 — from  
Genetic Risk to the Clinic*

October 6–8, 2021



[grandchallengesinpd.org](http://grandchallengesinpd.org)

## Wednesday, October 6, 2021

11:00 am Welcome Remarks

### SESSION 1: **LRRK2 AND GBA1 IN PARKINSON'S DISEASE**

SESSION CHAIR: José Brás, Ph.D., Van Andel Institute

**11:10 a.m.** Matt Farrer, Ph.D., University of Florida  
*Update on genetics/pathology of LRRK2-PD*

**11:40 a.m.** Ziv Gan-Or, M.D., Ph.D., Montreal Neurological Institute and Hospital  
*Update on genetics/pathology of GBA1-PD*

**12:10 p.m.** Mark Cookson, Ph.D., National Institutes of Health  
*Understanding genetic risk in sporadic PD*

**12:40 p.m.** Discussion

### SESSION 2: **LRRK2 AND GBA1 CELLULAR FUNCTION AND DYSFUNCTION I**

SESSION CHAIR: Michael Henderson, Ph.D., Van Andel Institute

**1:00 p.m.** Elisa Greggio, Ph.D., University of Padova  
*LRRK2 substrates and signaling pathways*

**1:30 p.m.** Erika Holzbaur, Ph.D., Perelman School of Medicine, University of Pennsylvania  
*LRRK2 in intracellular trafficking*

**2:00 p.m.** Sreeganga Chandra, Ph.D., Yale University  
*Lipid dys-homeostasis in GBA-linked Parkinson's disease*

**2:30 p.m.** Penny Hallett, Ph.D., McLean Hospital/Harvard Medical School  
*Models of GBA-linked alpha-synucleinopathy in Parkinson's disease*

**3:00 p.m.** Discussion

**3:15 p.m.** Introduction of keynote speaker

**3:20 p.m.** Ted Dawson, M.D., Ph.D, Johns Hopkins University  
Jay Van Andel Award for Outstanding Achievement in Parkinson's Disease Research  
*Translating the molecular pathogenesis of Parkinson's disease into new therapies*

**4:05 p.m.** Discussion



Thursday, October 7, 2021

**SESSION 3: *LRRK2* AND *GBA1* CELLULAR FUNCTION AND DYSFUNCTION II**

SESSION CHAIR: Darren Moore, Ph.D., Van Andel Institute

- 11:00 a.m.** Sabine Hilfiker, Ph.D., Rutgers, the State University of New Jersey  
*LRRK2 and endolysosomal pathway*
- 11:30 a.m.** Joe Mazzulli, Ph.D., Northwestern University Feinberg School of Medicine  
*GBA, Synuclein degradation, and lysosomal hydrolase trafficking*
- 12:00 p.m.** Emily Rocha, Ph.D., University of Pittsburgh  
*LRRK2 inhibition prevents endolysosomal deficits in Parkinson's disease*
- 12:30 p.m.** Abstract selected talk  
An Phu Tran Nguyen, Ph.D., Van Andel Institute  
*Common coding variants in LRRK2 associated with Parkinson's disease risk modulate LRRK2-dependent Rab10 phosphorylation*
- 12:45 p.m.** Discussion

**SESSION 4: THE ROLE OF NON-NEURONAL CELLS**

SESSION CHAIR: Gerhard (Gerry) Coetzee, Ph.D.,  
Van Andel Institute

- 1:00 p.m.** Andrew West, Ph.D., Duke University School of Medicine  
*LRRK2 and immune cells*
- 1:30 p.m.** Suzanne Pfeffer, Ph.D., Stanford School of Medicine  
*LRRK2 and Rabs: neurons and astrocytes*
- 2:00 p.m.** Inga Peter, Ph.D., Icahn School of Medicine at Mount Sinai  
*Link between Parkinson's and inflammatory bowel diseases*
- 2:30 p.m.** Abstract selected talk  
Céline Galvagnion, Ph.D., University of Copenhagen  
*Sphingolipid changes in Parkinson L444P GBA mutation fibroblasts promote  $\alpha$ -synuclein aggregation*
- 2:45 p.m.** Discussion
- 3:00 p.m.** Poster session via Slack



## Friday, October 8, 2021

### SESSION 5: CONVERGENCE OF PD RISK GENES

SESSION CHAIR: Patrik Brundin, M.D., Ph.D., Van Andel Institute

- 11:00 a.m.** Dimitri Krainc, M.D., Northwestern University  
*The interplay of mitochondrial and lysosomal dysfunction in Parkinson's disease*
- 11:30 a.m.** Michael Henderson, Ph.D., Van Andel Institute  
*Effects of LRRK2 on neuropathology*
- 12:00 p.m.** Richard Wade-Martins, M.A., D.Phil. University of Oxford  
*LRRK2 and GBA1 in the autophagic lysosomal pathway*
- 12:30 p.m.** Abstract selected talk  
Michaela E. Johnson, Ph.D., University of South Australia  
*Heterozygous GBA D409V mutations do not exacerbate pathological  $\alpha$ -synuclein spread in the preformed fibrils prodromal model in young mice*
- 12:45 p.m.** Discussion

### SESSION 6: CLINICAL TRIALS: PROMISE AND CONSIDERATIONS

- 1:00 p.m.** Introduction  
Panel Chairs: Shalini Padmanabhan, Ph.D.,  
The Michael J. Fox Foundation, and Roy N. Alcalay, M.D.,  
M.S., Columbia University
- LRRK2 Therapeutics*
- 1:05 p.m.** Joseph Lewcock, Ph.D., Denali  
**1:15 p.m.** Warren Hirst, Ph.D., Ionis/Biogen  
**1:25 p.m.** Matthew Fell, Ph.D., Merck  
**1:35 p.m.** Alastair Reith, Ph.D., GlaxoSmithKline Pharmaceuticals
- GBA Therapeutics*
- 1:45 p.m.** Olga Uspenskaya, M.D., Ph.D., Prevail  
**1:55 p.m.** S. Pablo Sardi, Ph.D., Genzyme/Sanofi  
**2:05 p.m.** Anthony Schapira, M.D., D.Sc., FRCP, FMedSci, University  
College London  
**2:15 p.m.** Peter Lansbury, Ph.D., Bial Biotech
- 2:25 p.m.** Panel discussion
- 2:45 p.m.** Closing remarks  
Darren Moore, Ph.D., and Michael Henderson, Ph.D.

