



LBSL Patient & Family Conference

JULY 24-26, 2026

Agenda

All times are listed in EST (UTC - 5). Topics and times are subject to change.

Friday, July 24

- 2:00 – 2:30 p.m. Introductions
***Ali Fatemi, MD, MBA**, Kennedy Krieger Institute*
***Michael McGinn**, Cure LBSL & Deos Therapeutics*
***Morgan Voigt**, Cure LBSL*
- 2:30 – 3:30 p.m. LBSL Natural History and Genetic Prognostic Factors
***Amena Smith Fine, MD, PhD**, Kennedy Krieger Institute*
Shannon Connelly, MS
- 4:00 – 5:00 p.m. Virtual Happy Hour

Saturday, July 25

- 12:00 – 1:00 p.m. Patient & Caregiver Support Groups
- 1:00 – 2:00 p.m. LBSL Gene Therapy: Our journey and next steps
***Christina Nemeth Mertz, PhD**, Kennedy Krieger Institute*

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Saturday, July 25 (continued)

- 2:30 – 3:30 p.m. Clinical Trials: What do they look like and feel like?
Amy Raymond, PhD, PMP, Worldwide Clinical Trials
- 3:30 – 4:00 p.m. LBSL Outcome Measures for Trials
Amena Smith Fine, MD, PhD, Kennedy Krieger Institute
Michelle Campbell, PhD, U.S. Food and Drug Administration
Morgan Voigt, Cure LBSL
- 4:00–5:00 p.m. LBSL and Gene Therapy Q&A Panel
Michelle Campbell, PhD, U.S. Food and Drug Administration
Ali Fatemi, MD, MBA, Kennedy Krieger Institute
Eric Mallack, MD, MBE, MS, Kennedy Krieger Institute
Amy Raymond, PhD, PMP, Worldwide Clinical Trials

Sunday, July 26

- 1:00 – 1:30 p.m. Magnetic Resonance Spectroscopy: How to use it for biomarker development in LBSL
Amena Smith Fine, MD, PhD, Kennedy Krieger Institute

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Sunday, July 26 (continued)

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| 1:30 – 2:30 p.m. | Taking Care of Ourselves: Panel on wellness, energy conservation and mental health
<i>Lisa Schafer, MD, Leipzig University Medical Center</i>
<i>Josh Beck</i> |
| 2:45 – 3:30 p.m. | From Surviving to Thriving: Navigating Physical Therapy and Rehabilitation When the Roadmap Doesn't Exist
<i>Jen Keller, MS, PT, Kennedy Krieger Institute</i>
<i>Jessica Fernandez, MD, Kennedy Krieger Institute</i> |
| 3:30 – 4:15 p.m. | Learning with LBSL: Panel on education and schools
<i>Beth McGinn, Co-founder, Cure LBSL</i>
<i>Stephanie Gilliam</i> |
| 4:15 – 4:45 p.m. | Patient Registries: Opportunities and why to participate
<i>Janine Lewis, MS, National Organization for Rare Disorders</i>
<i>Phil Yeske, PhD, United Mitochondrial Disease Foundation</i> |
| 4:45 – 5:00 p.m. | Closing Remarks |