

Rare Disease Fair & Summit

Fair: Friday, May 13, 2022
8:00 a.m. to 4 p.m.

Click to Join the Rare Disease Fair:

<https://seattlechildrens.webex.com/seattlechildrens/j.php?MTID=m8a005fd88c110919aaa03c8c893a0c34>

Meeting Number: 2456 595 7689

Password: fair

All times are listed in Pacific time

8:00 a.m. **Orientation to Webex**
Brittany Richey, *Manager, Administration, Seattle Children's Research Institute*

8:05 a.m. **Welcome**
Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*

NW Rare Disease Coalition

8:10 a.m. **NW Rare Disease Coalition Introduction**
Joshua Henderson, *Co-Founder, NW Rare Disease Coalition*

8:20 am **Gaps Identified from Regional Roundtable and Survey**
Max Brown, *Co-Founder, NW Rare Disease Coalition*

Diagnosis

8:35 a.m. **Introduction to Diagnosis**
Mason Gordon, *Rare Disease Advocate*

Moderator

Sarah Tompkins, *Ms. Wheelchair Washington USA 2022, Ehlers-Danlos Syndrome Advocate, RDLA Ehlers-Danlos Syndrome, Gastroparesis Syndrome, Rare Disease Advocate, Advisory Committee Member, & Advocacy Chair of Rare Disease Week 2021 & 2022*

8:40 a.m. **Fighting for Hope**
Mike Jackson, *Parent Advocate*

8:50 a.m. **How the RUSP Bill Will Advance Newborn Screening in WA**

Panelists:

My-Lin Thai, *Washington State Representative*
Monica Stonier, *Washington State Representative*

Moderator:

Max Brown, *Co-Founder, NW Rare Disease Coalition*

9:10 a.m. **WA Newborn Screening and State-to-State Collaboration Opportunities**

Panelists:

Dr. John Thompson, *Director of Newborn Screening, WA Department of Health*
Jessica Merritt, *Rady Children's Institute for Genomic Medicine*

Moderator:

Joshua Henderson, *Co-Founder of the NW Rare Disease Coalition*

9:45 a.m. **Break** (15 minutes)

Access

10:00 a.m. **Introduction to Access**

Lily King, *Rare Disease Advocate*

Moderator

Effie Parks, *Host, Once Upon a Gene*

10:05 a.m. **Reverse Engineering of Drug Development**

Monica Dudley-Weldon, *President/CEO/Founder SYNGAP1 Foundation*

10:15 a.m. **How RDAC Formation Will Advance Rare Disease Legislation in WA**

Panelist:

Jeff Holy, *Washington State Senator*

Moderator:

Max Brown, *Co-Founder, NW Rare Disease Coalition*

10:35 a.m. **Designing Mental Health Solutions to Address Gaps in Care**

Panelists:

Allison Fine, *Executive Director, The Center for Chronic Illness*
Cristal O'Loughlin, *Founder/CEO, Angel Aid Cares*
Dr. Albert Freedman, *Psychologist & Rare Dad, Freedman Counseling Associates*

Moderator:

Joshua Henderson, *Co-Founder, NW Rare Disease Coalition*

11:15 a.m. **Break** (20 minutes)

11:35 a.m. **Innovative Ways Pharma Puts Patients First**

Panelists:

Tara Flynn Zeilman, *Director, Product Access | Global Health & Social Impact, Pfizer Inc.*

Suzanne Tandon, *Sr. Director, Patient Advocacy, Vertex Pharmaceuticals*

Jessica Riviere, *Vice President, Global Patient Advocacy and Patient Engagement, Ultragenyx*

Keisha Robinson, *Associate Director of Clinical Research, Illumina*

Moderator:

Ken Gordon, *Executive Director, Northwest Association of Biomedical Research*

Commentator:

Tara Britt, *Founder and President, Rare Disease Innovations Institute Association Chair, North Carolina RDAC*

12:15 p.m. **Our Stories Rx: Turning Despair into Action, and Action into Hope**

Karen Ferguson, *Founder, Our Stories Rx*

12:30 p.m. **Access to Care and Genetic Testing: Learnings from Understudied Populations**

Dr. Juan Rodrigues-Flores, *Senior Manager, Regeneron Pharmaceuticals*

Treatment

12:45 p.m. **Introduction to Treatment**

Calvin Smith, *Rare Disease Advocate*

Moderator

Lindsey Topping-Schuetz, *Parent Advocate*

12:50 p.m. **Patient Story; Living the Life of Riley**

Katie Stevens, *Executive Director, Team Telomere*

1:00 p.m. **How Policy and Education Can Bring Cell and Gene Therapies to Patients Faster**

Panelists:

Dr. Jeff Chamberlain, *Professor in Departments of Neurology, Medicine, and Biochemistry University of Washington School of Medicine, McCaw Endowed Chair in Muscular Dystrophy*

Dr. Jennifer Hodge, *US Rare Neurology Medical Team Lead, Pfizer*

Kelly Maynard, *President, Little Hercules Foundation*

Jim McNally, *Chief Scientific Officer, BioAgilytix*

Moderator:

Tara Britt, *Founder and President, Rare Disease Innovations Institute, Associate. Chair, North Carolina RDAC*

1:40 p.m. **Until my son calls me "Dad": Re-inventing the drug development industry for one person**

Sanath Kumar, *Founder/CEO, Open Treatments Foundation*

1:55 p.m. **Break** (15 minutes)

Cardiovascular Scientific Rare Diseases

2:10 p.m. **Introduction to Cardiovascular**

Owen Schuetz, *Rare Disease Advocate*

Moderator

Ellen Morgan, *Founder/President, Pathways for Rare and Orphan Studies*

2:15 p.m.	Recognition and treatment of transthyretin amyloidosis Dr. Richard Cheng, <i>Associate Professor of Medicine (Division of Cardiology)</i>
2:35 p.m.	From Discussion to Design Shelley Bowen, <i>Founder and Director of Family Services, Barth Syndrome Foundation</i>
2:50 p.m.	Pediatric Cardiomyopathy Dr. Wendy Chung, <i>Departments of Pediatrics and Medicine, Columbia University</i>
3:10 p.m.	Noonan Syndrome Dr. Amy E. Roberts, <i>Co-Director, Center for Cardiovascular Genetics Conditions, Boston Children's Hospital, Associate Professor of Pediatrics, Harvard Medical School</i>
3:30 p.m.	NOTCH3 p.Arg1231Cys is Present in 1 in 92 Pakistani and Associated with Stroke Juan Rodriguez-Flores, <i>Senior Manager, Regeneron Pharmaceuticals</i>
3:50 p.m.	Closing Remarks Carolina Sommer, <i>Founder/CEO, Born a Hero Research Foundation</i>
3:55 p.m.	END

Rare Disease Fair & Summit

Summit: Saturday, May 14, 2022
8:15 a.m. to 3:50 p.m.

Click to Join the Rare Disease Summit:

<https://seattlechildrens.webex.com/seattlechildrens/j.php?MTID=m60123d3be92258dc2e8db5cc27960a0e>

Meeting Number: 2466 537 3222

Password: summit

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8:15 a.m. **Orientation to Webex**
Brittany Richey, *Manager, Administration, Seattle Children's Research Institute*

8:30 a.m. **Welcome**
Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*

Sharing and Turning Stories into Meaningful Impact

Moderator

Ellen Morgan, *Founder and President, Pathways for Rare and Orphan Studies*

8:40 a.m. **Your Voice Matters: How to Powerfully Write Your Story**
Jessica Lynn, *Patient Worthy, Writer*

9:10 a.m. **Finding, formulating and telling your rare disease stories through podcasts**
Effie Parks, *Host, Once Upon a Gene*

9:40 a.m. **How to Frame Your Talk with Legislators**
Max Brown, *Co-Founder, NW Rare Disease Coalition*

Lindsey Topping-Schuetz, *Parent Advocate*

10:10 a.m. **Our Stories Rx: From Diagnosis to Treatment—Navigating Healthcare Ecosystem**
Karen Ferguson, *Founder, Our Stories Rx*

10:40 a.m. **Composing and delivering compelling presentations**
Dr. Matt McGarrity, *Communications Professor, University of Washington*

11:40 a.m. **Share Your Rare: Speaking in Educational Settings**
Melissa McGowan, *Board Member, Newsletter Editor, Born a Hero Research Foundation*

12:10 p.m. **Break** (30 minutes)

Coping Strategies Workshops

Moderator

Jenny Sommer, *Seattle Rare Disease Fair, Assistant Coordinator, Born a Hero Research Foundation*

12:40 p.m. **Meditation**
Katie Stevens, *Executive Director, Team Telomere*

1:10 p.m. **Caring for the Caregivers: Sensory Self-Care in Micro-moments Per Day**
Cristal O'Loughlin, *CEO and Founder, Angel Aid Cares*

1:40 p.m. **Yoga**
Katie Stevens, *Executive Director, Team Telomere*

2:10 p.m. **Spirituality A Powerful Tool for Caregiving**
Sandy Powers, *Life Coach, Mindful Healing Junction*

Getting Involved

2:40 p.m. **Getting Involved With Young Adult Advocacy**
Eimile McKinnon, *Young Adult Representative of RDLA (YARR)*

3:10 p.m. **NW Rare Disease Coalition**
Joshua Henderson, *Co-Founder, NW Rare Disease Coalition*

3:25 p.m. **Expanding the Rural Rare Disease Community Project to WA and Beyond**

Panelists:

Tara Britt, *President, Rare Disease Innovations, Institute Associate Chair, North Carolina RDAC*
Khrystal D. Davis, *Founding President, Texas Rare Alliance*
Marissa Dunn, *Regional Director of Advocacy, Vertex Pharmaceuticals*
Carolina Sommer, *CEO and Founder, Born a Hero Research Foundation*

Moderator:

Joshua Henderson, *Co-Founder, NW Rare Disease Coalition*

3:45 p.m. **Final Remarks**
Carolina Sommer, *CEO and Founder, Born a Hero*

3:50 p.m. **END**

Thank you to our event sponsors!

