Rare Disease Fair & Summit

June 4th & 5th, 2021

Rare Disease Fair
June 4th, 8:45 a.m. to 2:30 p.m.

All times are listed in Pacific time

8:45 a.m.	**Join the Event** Webex Meeting ID: **133 426 5771** Password: **rarefair**
8:45 a.m.	Orientation to Webex Brittany Richey, *Manager, Administration, Seattle Children's Research Institute*
9:00 a.m.	Welcome Dr. Eric Tham, *Interim Senior Vice President, Seattle Children's Research Institute*
9:05 a.m.	An Introduction Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*
Rare Disease Advisory Council	
9:15 a.m.	Introductions by Tyler Downs, *Pfeiffer Syndrome Teen; Patient Advocate*
9:20 a.m.	NW Rare Disease Coalition and the Region's Rare Disease Community Needs, Roundtable Moderator: Tara Britt, *Founder and President, Rare Disease Innovations Institute, Assoc. Chair, NC RDAC* Panelists Dr. Michael Bamshad, *Professor and Chief, Division of Genetic Medicine, Allan and Phyllis Treuer Endowed Chair in Genetics and Development, Department of Pediatrics, University of Washington and Seattle Children's Hospital* Dr. Jeff Chamberlain, *McCaw Endowed Chair in Muscular Dystrophy, Professor, Departments of Neurology, Medicine and Biochemistry, University of Washington School of Medicine, Director of the Senator Paul D. Wellstone Muscular Dystrophy Specialized Research Center* Dr. Katrina Dipple, *Professor and Associate Chief and Medical Director, Division of Genetic Medicine, Department of Pediatrics, University of Washington and Seattle Children's Hospital. Principal Investigator, Seattle Children's Research Institute* Io Dolka, *Founder, Managing Director & Chief Care Advocate, GreyZone Health* Dr. Ian Glass, *Medical Genetics; Principal Investigator, University of Washington Birth Defects Research Laboratory and Seattle Children's Research Institute* Joshua Henderson, *Head of Rare Diseases, Pulse Infoframe* Dr. Richard James, *Principal Investigator, Seattle Children's Research Institute* Charles Sang, *Biotech Executive (Retired)* Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation* John Thompson, *Director of the Newborn Screening Program, Washington State Department of Health*
10:00 a.m.	NW Rare Disease Coalition and the Region's Rare Disease Community Needs, Discussion and Q&A

10:10 a.m.	**Patient Story** Ben Kjar, *Crouzon Syndrome Advocate, 3-time State Wrestling Champion, 4th place in 2011 Wrestling National Championship*
10:25 a.m.	**Door Prize Drawing**
10:30 a.m.	*Break*

Rare Disease Policy

10:40 a.m.	Introductions by Grace Anto, *Teen, Patient Advocate, Frontonasal Dysplasia*
10:45 a.m.	**Rare Disease Policy, Roundtable** Moderator: Hugh Ewart, *Senior Director, State and Federal Government Relations, Seattle Children's* Panelists **Max Brown**, *Principal Consultant and Lobbyist, Desimone Consulting Group* **Jessie Conta**, *Manager, PLUGS® & Laboratory Stewardship Program, Seattle Children's* **Alyss Patel**, *State Policy Manager, Western Region, NORD* **Dean Suhr**, *President/Founder, Rare Army; Co-Founder, MLD Foundation* **Jamie Sullivan**, *Director of Public Policy, EveryLife Foundation for Rare Diseases* **Saira Sultan**, *Policy Consultant, Haystack Project; President/CEO- Connect 4 Strategies*
11:25 a.m.	**Rare Disease Policy, Discussion and Q&A**
11:35 a.m.	**Patient Story** Jono Lancaster, *Treacher Collins Advocate, Co-Founder, Love Me, Love my Face Foundation*
11:50 a.m.	*Break*

Scientific Talks

12:30 p.m.	Introductions by David Sommer, *Vice President, Born a Hero, Research Foundation*
12:30 p.m.	**Modeling Dynamic Upper Airway Obstruction in Patient's with Robin Sequence: Implications for Surgical Planning** Dr. John Dahl, *Otolaryngologist, Seattle Children's Assistant Professor in the Department of Otolaryngology, Head and Neck Surgery at the University of Washington*
12:50 p.m.	**How Activin A Became a Therapeutic Target for Fibrodysplasia Ossificans Progressive (FOP)** Dr. Aris Economides, *Vice President, Research, Regeneron Pharmaceuticals*
1:10 p.m.	**Finding Novel Mechanisms and Therapies for Fibrous Dysplasia (FD) and Fibrodysplasia Ossificans Progressiva (FOP)** Dr. Edward Hsiao, *Associate Professor, University of California – San Francisco*
1:30 p.m.	**Bones and Joints in Rare Diseases: A Guide to Orthopedic Care** Dr. Klane White, *Director, Skeletal Health and Dysplasia Program, Seattle Children's; Principal Investigator, Seattle Children's Research Institute*
1:50 p.m.	**Cure, Support and Advocate: Connecting with the MPS Community** Leslie Urdaneta, *Director of Family Support and Communication, National MPS Society*
1:55 p.m.	**TBD** Dr. Ethylin Jabs, *Vice Chair, Department of Genetic and Genomic Sciences, Icahn School of Medicine, Mount Sinai Medical Center*
2:15 p.m.	**Door Prize Drawing**

2:20 p.m.	Final remarks Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*
2:30 p.m.	Adjourn

Rare Disease Summit
June 5th, 8:45 a.m. to 3:00 p.m.

All times are listed in Pacific time

8:45 a.m.	**Event Access Link: Join the Event** Webex Meeting ID: **133 340 6244** Password: **raresummit**
8:45 a.m.	Orientation to Webex Brittany Richey, *Manager, Administration, Seattle Children's Research Institute*
9:00 a.m.	Welcome Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*
Policy	
9:10 a.m.	Introductions by Melissa McGowan, *Board Member and Newsletter Editor, Born a Hero Research Foundation*
9:10 a.m.	How Rare Patients and Caregivers Might be Impacted by Federal Healthcare Policy Changes in 2021 Max Brown, *Principal Consultant and Lobbyist, Desimone Consulting Group*
9:25 a.m.	The Power of One Dean Suhr, *President and Founder, Rare Army; Co-Founder, MLD Foundation*
9:45 a.m.	Advocacy Opportunities at the EveryLife Foundation for Rare Diseases Lindsey Cundiff, *Associate Director of Patient Engagement, EveryLife Foundation for Rare Diseases*
Registries	
10:00 a.m.	Research Registry Roundtable Moderator: Ellen Morgan, *Founder& President Pathways for Rare and Orphan Studies (PROS) Foundation* Panelists **Matthew Caffet**, *Strategic Operations Manager, Genetic Alliance, PEER Registry* **Stephanie Christopher**, *Associate Director, Research Programs, NORD* **Joshua Henderson**, *Head of Rare Diseases, Pulse Infoframe*
Resources	
10:30 a.m.	Patient-Centered Rare Disease Clinical Trials: The Future is Here Scott Schliebner, *Senior Vice President, Scientific Affairs and Therapeutic Expertise, Center for Rare Diseases, PRA Health Sciences*
10:45 a.m.	Tale of a Troubled Clinical Trial Ellen Morgan, *Founder/President PROS Foundation*
11:00 a.m.	PNW Undiagnosed Diseases Network Clinical Site Dr. Katrina Dipple, *Associate Division Chief, Director, Medical Genetics; Principal Investigator, Seattle Children's Research Institute*
11:15 a.m.	How to Get Lab Tests Covered by Insurance: A PLUGS® Toolkit Jessie Conta, *Manager, PLUGS® & Laboratory Stewardship Program, Seattle Children's*

11:35 a.m.	Door Prize Drawing
11:40 a.m.	*Break*
Support	
12:20 p.m.	Supportive Resources for the Chronic Illness and Rare Disease Communities Allison Fine, *Founder & Executive Director, The Center for Chronic Illness*
12:35 p.m.	An introduction to NAMI's Peer Support Services in Washington Lauren Simonds, *Executive Director& CEO, National Alliance on Mental Illness, Washington State*
12:50 p.m.	Using Storytelling as a Way to Raise Awareness Effie Parks, *Founder & Host, Once Upon A Gene Podcast*
1:05 p.m.	Supporting Men Who Have a Child in Their Life with Special Health Care Needs: The WA State Fathers Network Louis Mendoza, *Manager, Washington State Fathers Network, Kindering*
1:20 p.m.	Leave Your Baggage at the Door. How One Hour Can Change Your Life. Jennifer Pratt and Heather Heyer, *Dance Instructors, Journey Fitness REFIT*
Community	
1:35 p.m.	FAM177A1 Disruption. What's Your Function? Jill Hawkins, Parent, FAM177A1 Advocate, Charlotte and Cooper's diagnostic quest
1:50 p.m.	No One Gets Left Behind Mike Jackson, *Parent Advocate, CLN2 Batten Disease, Evelyn's Law for Genetic Testing*
2:05 p.m.	Stories from the Front Lines of Advocacy & Innovation in the WA Rare Community Moderator: Max Brown, *Principal Consultant and Lobbyist, Desimone Consulting Group* Panelists **Mary McDirmid**, *Founder, Plan ABZ Special Needs Planning; Managing Director, MassMutual NW* **Sanath Ramesh**, *Founder and CEO, OpenTreatments Foundation* **Sarah Tompkins**, *Advisory Committee Member, RDLA; Ms. Wheelchair Pacific Coast USA, 2021*
2:35 p.m.	Door Prize Drawing
2:40 p.m.	Summary Joshua Henderson, *Head of Rare Diseases, Pulse Infoframe*
2:50 p.m.	Final Remarks Carolina Sommer, *Founder and CEO, Born a Hero Research Foundation*
3:00 p.m.	Adjourn

We are grateful for the kindness and support of our **generous sponsors.**
Thank you for helping us accelerate research for rare diseases!