



2021

**RARE DRUG DEVELOPMENT
SYMPOSIUM – A VIRTUAL EVENT**

Navigating Your Virtual Event Experience

Pre-Conference Workshop – June 9, 2021

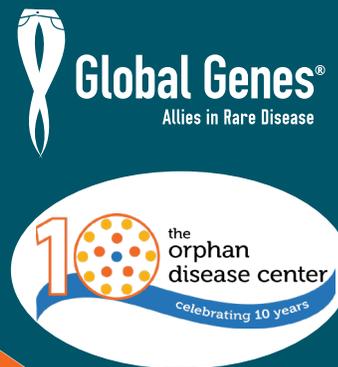
12:00 PM ET – 2:30 PM ET

Main Program – June 10-11, 2021

10:00 AM ET – 5:00 PM ET (Day 2 ends 4:00 pm ET)

globalgenes.org/event/rare-disease-drug-development/

#RDDS





RARE DRUG
DEVELOPMENT SYMPOSIUM

Welcome to the **2021 RARE Drug Development Symposium**, in partnership with the Orphan Disease Center of the University of Pennsylvania, happening virtually **June 9-11, 2021**.

We encourage the rare disease community to explore their role in the rare disease drug development process in new and innovative ways at this year's symposium. From targeted breakouts to dedicated office hours with subject matter experts, our goal is to help you discover your role in advancing drug therapies and educating yourself on the complex world of rare disease drug development.

To help you get the most out of your virtual event, we have created this simple guide. But as always, if you have additional questions, please do not hesitate to reach out to our team at events@globalgenes.org.

Table of Contents

About Global Genes and the Orphan Disease Center	4
How to Access the Event	5
Create Your Own Agenda	5
Manage Your Profile & Contact Information	5
How to Access Live Sessions	6
Networking	7
One-On-One Messaging	7
Send a Message	7
How to Navigate the Event	8
Tips for a Successful Virtual Experience	9
Thank You	10

About Global Genes®

Global Genes is a 501(c)(3) non-profit organization dedicated to eliminating the burdens and challenges of rare diseases for patients and families globally. In pursuit of our mission we connect, empower, and inspire the rare disease community to stand up, stand out, and become more effective on their own behalf -- helping to spur innovation, meet essential needs, build capacity and knowledge, and drive progress within and across rare diseases. We serve the more than 400 million people around the globe and nearly 1 in 10 Americans affected by rare diseases. If you or someone you love has a rare disease or are searching for a diagnosis, contact Global Genes at 949-248-RARE or visit our resource hub at www.globalgenes.org.

DONATE TODAY: Text RARE to 41444
Visit <https://globalgenes.org/donate-now>

FOLLOW US:
[@globalgenes](https://www.instagram.com/globalgenes)



About the Orphan Disease Center

Mission

The Orphan Disease Center will develop transformative therapies using platform technologies that can be deployed across multiple rare diseases. We will emphasize disorders with substantial unmet need independent of their incidence and will strive to assure access to patients of all populations.

Impact

Each type of orphan disease affects such a small subset of the population, so the need for research and funding in this area is largely unmet. Our Center, the first of its kind, works closely with patient groups and foundations, pharma and biotech, and the academic community. We bring a unique set of programs to the table, enabling us to add value at any stage - from building the initial knowledge base to enabling therapeutic development. Through our grants, Programs of Excellence, JumpStart programs, and a number of new initiatives, the ODC seeks to drive therapeutic development for rare diseases. We help identify and fund the most promising therapeutics while also tackling obstacles present in rare disease drug development.

orphan-disease-center.med.upenn.edu

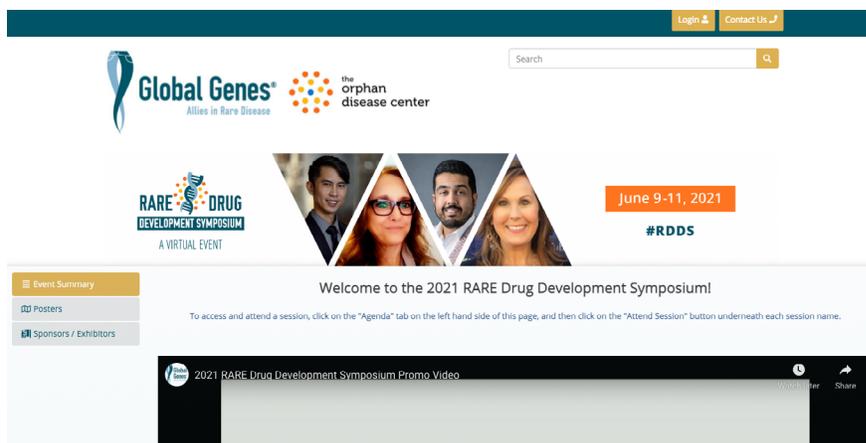
Follow Us: [@MDBRide4Rare](https://www.instagram.com/MDBRide4Rare)



VIRTUAL EVENT PLATFORM

The virtual event platform is where you can access everything you need to participate in RARE Drug Development Symposium. To access the virtual event platform, please enter the following URL into your web browser [here](#)

How to Access the Event



NOTE: You will need to create a new account the first time you log-in by clicking on the “Login” button in the top right corner. Please remember and/or save your email and password you use to create your account, as you will need that information to log-in to the virtual event platform throughout the event.

Create Your Own Agenda

Adding sessions to your customized itinerary for the event

1. To edit your schedule, click on the agenda icon. There will be two tabs, one that shows all sessions and one that only shows the sessions selected by you for your personal symposium itinerary.
2. If you want to add a session to your itinerary find the session you would like to add and click on the green “add to my itinerary” plus button to the right of the session title.
3. If you want to remove a session, click on the red “remove from itinerary” minus button to the right of the session title.

Manage Your Profile

How much you share about yourself with other attendees is up to you. You can update your information as well as hide your contact information or make it visible to others.

1. Access your profile settings by clicking “My Account” at the top of the page to see your profile.
2. When you enter the platform, you will be able to update your attendee profile.
3. You can update your profile at any time by accessing your profile settings.

Choose Whether to Share Your Information with Other Attendees

1. Click on the Attendees icon to opt out.
2. Go to the "Attendee List" tab on the left hand navigation bar where you can opt in/opt out.

How to Access the Live Sessions

Each session opens 15 minutes prior to the start time. To access your live streamed session(s) click the "My Itinerary" or "Agenda" tab to view the schedule that you created and launch from there or click on the "Sessions" tab and use the Filter by Date or Tag to locate and select your desired sessions.

Can I interact during sessions?

While watching or listening, you can easily switch between these custom features:

- **Session Details** – Review session description and speaker biographies
- **Join The Conversation** – Located on the top right hand corner of your screen, click on JOIN THE CONVERSATION to interact with other participants
- **Live Chat** – During sessions, interact and network with other online attendees and event moderators
- **Resources** – Download presentation materials
- **Session Notes** – Easily jot down key take-aways from the session you are viewing and receive them automatically via email once the session ends
- **Private Message** – Send a private message during a live chat session to fellow online attendees
- **Request Live Help** – Our staff is always there to help if you run into any issues with your online experience

NETWORKING

In order to engage in Networking, please make sure you complete “My Account” at the top of the home page. Then feel free to post on our Social Wall.

One-On-One Messaging

A live chat feature is available within the virtual event platform during each session.

Send a Message

1. Tap on the Attendees tab.
2. Find the person you would like to message and tap on their name.
3. Write your message, then tap Send.

Social Wall

To give you an opportunity to network with the larger group of attendees, we are offering you networking through the Social Wall. The tab to enter the Social Wall is located on the left side of your screen.

Join The Conversation

1. Resides on the right-hand side of the event page
2. Allows for one general chat thread

HOW TO NAVIGATE THE EVENT

☰ Event Summary	→	Description of the event
📅 Agenda	→	Agenda, Sessions, and Your Personal Itinerary
👤 Meet the Speakers	→	Presenter Bios and Photos
📄 Posters	→	New Research abstracts and posters
👤 CureAccelerator Live!	→	Access special event, in partnership with Cures Within Reach
🏪 Sponsors / Exhibitors	→	Visit exhibitor booths to achieve badges
📄 Session Resources	→	Get handouts and meeting materials from the sessions. <i>Not all sessions will have handouts</i>
👥 Attendees	→	See who else is at the meeting and join the conversation to chat
📷 Social Wall	→	Post a picture, comment, or just say hi
🏆 Achievements and Badges	→	Participate in sessions and achieve badges
❓ FAQs	→	Get answers to your questions about this meeting and platform
👉 About Global Genes	→	Learn more about Global Genes
🏠 About Orphan Disease Center	→	Learn about the Orphan Disease Center
⚠️ System Checker	→	Use this if your system is not syncing with the platform
🗣️ Technical Support	→	Contact us if you are having technical difficulties
📺 Archives	→	Access recordings of the live sessions here

TIPS FOR A SUCCESSFUL VIRTUAL EXPERIENCE

We know that attending a virtual event can be an adjustment for participants. We have compiled a list of tips to help you get the most out of your virtual event experience.

- **Test Your Tech:** Before the event, make sure that your chosen device is working properly, fully charged, and has wifi access. We will do everything on our end to ensure a great experience and we don't want you to miss a thing. If you experience an issue, please email us at events@globalgenes.org.
- **Connect with Others:** We encourage you to take advantage of networking tools to see who is participating and connect with them. This will help you to get more out of your experience while also growing your personal network.

If you are active on social media, follow the event hashtag – #RDDS. We'd love to have you join the conversation as we post about key takeaways, event highlights and more. Tag us in your posts!

Don't let the networking stop when the event stops. We encourage you to trade contact information and connect with fellow participants on other platforms such as LinkedIn or Facebook or simply by email.

- **Ask Questions:** The event offers many opportunities to engage with the content and get your questions answered by the presenting experts. This event is meant to help you foster collaborative and cross-functional learning and break down knowledge silos, so don't be shy about taking advantage of this moment to learn everything you can. We want you to come out of this being able to apply what you've learned immediately.
- **Minimize Distractions:** When attending a virtual event, it can be easy to start multi-tasking and become distracted. To get the most out of the sessions, close your email and block off your calendar so you can focus on the presenters and engage in the Q&A opportunities.
- **Flex Your Engagement Muscles:** Add a little competition to your virtual event experience by flexing your engagement muscles – as you explore the app, connect with peers and engage in conversations, you'll earn points and achievements. You can even opt in to compete on the leaderboard against other event attendees. You never know who will be right on your heels! Attendees will have the chance to win either a complimentary registration to the Global Genes 2021 RARE Patient Advocacy Summit or the 2022 RARE Drug Development Symposium.

Thank You

to our 2021 RARE Drug Development Advisory Committee

Thank you to the rare disease leaders and advocates who have contributed to shaping the agenda content! We appreciate the ideas and insights that you have shared with us and the community.

Lara Bloom, Professor, President & CEO, The Ehlers-Danlos Society

Kristina Bowyer, Ph.D., Vice President, Patient Centric Drug Development at Ionis Pharmaceuticals, Inc.

Samantha Charleston, Director, Programs and Community Engagement, Orphan Disease Center, Perelman School of Medicine, University of Pennsylvania

Benjamin Forred, MBA, Director, Clinical Research - Genetics & Genomics and the CoRDS Registry, Sanford Research

Sumathi Iyengar, M.D., Co-Founder and Executive Director, Wiskott-Aldrich Foundation

Jenny Klein, M.S., Co-Founder, Mucopolidosis Collaborative Research Network

Rob Long, Executive Director, Uplifting Athletes Inc.

Eric Marsh, M.D., Ph.D., Clinical Director- Orphan Disease Center Associate Professor of Neurology and Pediatrics, Perelman School of Medicine; University of Pennsylvania

Debbie Requesens, Ph.D., Director, Jumpstart, Orphan Disease Center, Perelman School of Medicine, University of Pennsylvania

Kari Rosbeck, President and Chief Executive Officer, Tuberous Sclerosis Alliance

Rodney Samaco, Ph.D, Assistant Professor & Investigator, Dept of Molecular and Human Genetics, Baylor College of Medicine

Shoshana Shendelman, Ph.D., CEO & Founder, Applied Therapeutics

Christe Wekon-Kemeni, M.D., Resident Physician, Department of Pediatrics, UNC Children's Hospital

This guide is proudly sponsored by

GOLD



SILVER



BRONZE





28 Argonaut, Suite 150
Aliso Viejo, CA 92656

+1-949-248-RARE (7273)

Follow us:

www.globalgenes.org

@globalgenes



©Global Genes 2021. All rights reserved.



University of Pennsylvania
TRL Suite 1200
125 South 31st Street
Philadelphia PA 19104

Follow us:

orphandiseasecenter.med.upenn.edu

@MDBRide4Rare

