The conduct of clinical research in rare diseases is essential.

5th Conference on Clinical Research for Rare Diseases

November 19, 2018

Hilton Rockville Hotel & Executive Meeting Center
Rockville, MD

Despite their rarity, many fundamental advances in medicine have come from the study of rare diseases and these have benefited common diseases. In order to assure the future of this research, the training of the next generation of investigators in this field is important.

Who Should Attend:

New investigators, trainees, junior faculty, and others interested in rare disease research methodology.

Abstract & Poster Session:

Attendees will have the opportunity to present their work through submission of an abstract and inclusion of a poster session; investigators working on rare diseases will provide feedback and advice.

In addition, attendees will:

- Meet key leaders in rare diseases research.
- Engage in formal and informal networking and idea-generation with other trainees and investigators.
- Discuss career development and strategic planning for academic success.
- Explore research collaborations.

Registration & Travel Awards:

Registration is $100 and includes: meals, refreshment breaks, and meeting materials.

Travel awards of $750 are available on a competitive basis.

For full program details, registration material, and travel award application please visit:

www.rdcrn.org/conference

sponsored by: Rare Clinical Diseases Research Network
Initiative of the National Center for Advancing Translational Sciences (NCATS)

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Morning Session	***Lecture Title/Event***	***Summary***
7:00 – 8:00	*Breakfast, Registration, and Poster Set-up*	
8:00 – 8:10	*Welcome and Introduction*	Review agenda, introductions
8:10 – 8:30	*"Challenges and solutions to conducting clinical research in rare diseases"*	Outline of major issues with additional intent of setting up the day
8:30 – 9:15	*"Clinical trial design issues and options for study of rare diseases"*	Studies of small sizes; trial design (open-label, multicenter, N-of1), etc
9:15 – 9:45	*"Considerations for the use of biomarkers in the study of rare diseases"*	Discussing the unique issues of utilizing biomarkers as study outcomes in research in rare diseases
9:45 – 10:00	*Break*	
10:00 – 11:00	*How to work with industry and the FDA for rare diseases research*	Representatives from biomedical industry and the FDA outline the many complex issues involved in such collaborations
11:00 – 11:30	*Importance of investigator and patient advocacy group (PAG) Collaborations*	2 Investigators, and 2 CPAG partners discuss these critical interactions and partnerships
11:30 – 12:30	*Highlighting novel/award-winning abstracts*	Poster presentations with panel input/critique
12:30 – 12:45	*Buffet lunch pick-up*	
12:45 – 2:00	*Poster session through lunch*	
Afternoon Session		
2:00 – 3:15	*Breakout session 1*	• Career development and mentoring ***OR*** • Practical logistics of conducting research in rare diseases
3:15 – 3:30	*Break*	
3:30 – 4:45	*Breakout session 2*	• Career development and mentoring ***OR*** • Practical logistics of conducting research in rare diseases
5:00 – 5:30	*Critical evaluation of day's program*	Written (anonymous) evaluation form with comments; open discussion
Evening Session		
5:30 – 7:00	*Wine and cheese reception and additional poster discussion*	Informal session Trainee networking