2019 Spring Newsletter

A Bustling Year Ahead

I-ACT for Children is bustling with activity this spring, as we plan for three public meetings in 2019, design and launch our Quality Improvement initiative and welcome three new Members to our organization.

We marked the first part of the year with substantial growth: Eli Lilly and Company joined I-ACT for Children as our newest Sustaining Member, while Bayer and Chiesi Farmaceutici joined as our newest Annual Members. We are so proud to be working with these companies – as well as Sustaining Members Johnson & Johnson and Novartis – to accelerate and enhance the quality of pediatric clinical trials.

Earlier this month, Collin Hovinga, our SVP for Clinical & Scientific Development, presented a poster at the Muscular Dystrophy Association's annual conference on our work with Parent Project Muscular Dystrophy to design a platform trial to study potential Duchenne muscular dystrophy therapies. Collin also presented at a meeting organized by the Society of Clinical Research Associates (SoCRA) that detailed the pediatric device development expertise within our I-ACT for Children Site Network.

We launched the design phase of our Quality Improvement initiative with an Expert Meeting in March; are preparing for a meeting with the FDA and the Duke Clinical Research Institute (DCRI) that will explore what therapies may be needed to address the youth vaping epidemic; and continue to build our pediatric research site network, including efforts to become interoperable with our ex-US "sister networks" such as connect4children (Europe) and MICYRN (Canada).

You can read more about many of these efforts below. I can't wait to share more with you in future newsletters as our 2019 initiatives progress!

Laura Gordon
Chief Executive Officer

May 15 Meeting with FDA, DCRI will Address E-Cigarette Epidemic in Youth

E-cigarette use among youth/adolescents has become a serious issue in the United States, but there is a dearth of information about what therapies may be effective in treating e-cigarette addiction in this age group. I-ACT for Children is hosting a meeting on May 15 with the **Duke Clinical Research Institute** and the **U.S. Food & Drug Administration** that will explore the unique challenges faced in addressing the problem. Experts at the meeting will discuss the differences in how addiction works in the adolescent brain, youth misperceptions about the dangers of e-cigarettes versus tobacco use and the distinct messages and communications channels that influence this population.

The goal of the meeting is to understand what is known about cessation therapies in adolescents, identify the gaps in information and discuss where resources should be focused as researchers and other experts seek answers for how best to treat e-cigarette addiction in this vulnerable age group. We are expecting several hundred people to attend the meeting, either in person or via webcast.

Anyone interested in attending the meeting (in person or via webinar) can register using the following link: https://www.eventbrite.com/e/youth-tobacco-cessation-science-and-treatment-strategies-tickets-56911369438.

Our US Site Network is 44 Strong...

... and it continues to grow!

The I-ACT Site Network now includes 44 sites with executed contracts. You can visit our updated site-network page (https://www.iactc.org/i-act-for-children-site-network/) and use our interactive map for information on specific sites. We also are working with conect4children (c4c) and MICYRN to develop interoperability agreements that will provide our Members with access to a global research network.

Expert Meeting Helps I-ACT Site Network QI Initiative Take Shape

Last month, we hosted an Expert Meeting in Chicago to finalize the design of our Quality Improvement initiative. The goal of this effort is to apply QI metrics to the pediatric clinical trials conducted within the I-ACT for Children Site Network. These metrics will be designed to maximize both the quality and speed of pediatric clinical trials through refined processes, pediatric-specific training, network information sharing and other efforts. The QI initiative is led by I-ACT for Children's QI expert Carol Rosenberg, ND, DNP, RN, and SVP Lisa Benson, BS,

CCRP, CRCP. Our key partners in the effort are Peter Margolis, MD, PhD, and his team from the James M. Anderson Center for Health Systems Excellence.

More than 40 experts in the pediatric clinical trial and QI fields attended the hands-on, two-day meeting to discuss the QI initiative and provide input on its measures. Later this year, we will launch a pilot version of the initiative and roll out our first cohort of sites.

I-ACT for Children CEO Laura Gordon welcomes expert meeting attendees by describing the positive impact QI metrics will have in pediatric clinical trials.

I-ACT for Children, PPMD Partner on Platform Trial

Today, most clinical trials are designed to compare one compound to a placebo in a given patient population. But when studying therapies for some diseases and patient populations, this

traditional study design can result in difficulty enrolling enough patients in a study and/or long waits for even one trial to be completed.

These challenges are a reality for children with Duchenne muscular dystrophy (DMD). In keeping with I-ACT for Children's mission of using innovative approaches to enhance and accelerate pediatric trials, we are working with Parent Project Muscular Dystrophy (PPMD) to launch a platform trial for DMD. The platform trial will be designed to study multiple drugs in multiple patient types at any given time, with one common placebo-controlled arm. Platform trials also can be designed so that if a trial arm with a compound that is not showing benefit is shut down, patients can be efficiently randomized to other trial arms. These types of features are critical when addressing a progressive and debilitating disease such as DMD.

I-ACT for Children and PPMD are working with the Critical Path Institute and Berry Consultants to draft the study protocol and are building a consortium that will operationalize and launch the trial. The consortium will host a public meeting this fall to seek input on the draft protocol from DMD stakeholders (researchers, advocates and families, drug sponsors and regulatory agencies); stay tuned for future meeting details.

I-ACT for Children Awards its First Travel Grants

Congratulations to **Sara Kramer, MPH, CCRP,** and **Deepak Chellapandian, MD**, who were awarded the inaugural I-ACT for Children Travel Grants.

The I-ACT for Children Travel Grant Program supports the continuing education of those involved in pediatric clinical trial research. The grants support attendance at a relevant research conference of an early-career Investigator; study nurse or coordinator; or research administrator/research administration staff who are involved in pediatric clinical trials.

Two $5,000 grants are awarded in January of each year. To apply, visit https://www.iactc.org/travel-grant-program/. We will begin accepting applications for the next awards in Q3 2019.

Sara Kramer, MPH, CCRP, will use the award to attend the 2019 SoCRA Pediatric Clinical Trials Conference. Ms. Kramer is a clinical research coordinator and a key member of the pediatric clinical research team at the University of Minnesota.

Dr. Deepak Chellapandian will use the award to attend the 45th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT). He is an early-career investigator within Johns Hopkins All Children's Cancer and Blood Disorders Institute with expertise in the design and conduct of early-phase trials in hematopoietic stem cell transplantation for children with non-malignant disease.

Career Opportunities

We are looking for self-motivated people to join our team! If you are interested in working with us to improve pediatric trials, visit - https://www.iactc.org/careers/

Copyright © 2019 Institute for Advanced Clinical Trials for Children, All rights reserved.

You are receiving this email as a member of the I-ACT for Children site network.

Our mailing address is:

Institute for Advanced Clinical Trials for Children

9211 Corporate Blvd Ste 260

Rockville, MD 20850-3856

Add us to your address book

Want to change how you receive these emails?
You can update your preferences or unsubscribe from this list.