A Busy Year, an Exciting Future

When I look back on all that we have accomplished this year at I-ACT for Children, I can’t quite believe it. In the spring, we helped coordinate a meeting for FDA that discussed the research gaps in treatments to address the e-cigarette epidemic among youth. This fall, we convened 85 experts to explore how to maximize the success of including adolescents in adult clinical trials, with the goal of accelerating research in this “older” pediatric population. Working with Parent Project Muscular Dystrophy and a multi-stakeholder team of clinical trial experts, we’re designing a platform trial that has the potential to more quickly and efficiently test investigational treatments for Duchenne muscular dystrophy. And working with our pharmaceutical-company members, we are leading a variety of projects to introduce both innovation and efficiency into their efforts to enhance and accelerate the development of safe and effective medicines for children.

Based on the solid foundation 2019 has provided, 2020 promises to be even more fruitful. Our wonderful, hard-working staff continues to grow, and in January, we will welcome our first Chief Medical Officer. I am thrilled to announce that Gary Noel, MD, will join us starting on Jan. 6, after a long tenure with Johnson & Johnson and roles with other pharmaceutical companies as well as an
esteemed academic career. Dr. Noel takes the reins from Ed Connor, MD, MBE, who helped found I-ACT for Children, chairs our Board of Directors and was kind enough to serve as Interim CMO while we sought someone to take on the role full time.

As we enter this season of thanksgiving and reflection, I thank you for your ongoing support – whether that’s through working on projects together, being a member and/or donor to I-ACT for Children or just good old-fashioned cheering us on from the sidelines. Our mission truly fuels us every day as we work to improve therapeutic options for children. But knowing that all of you are right there by our side makes the work even more satisfying.

We still have much to do before we head out for the holidays. But let me take this moment to wish you all happiness in the New Year. I look forward to working together in 2020!

Laura Gordon
Chief Executive Officer
Platform Trial Stakeholder Meeting Brings Duchenne Community Together to Discuss Innovative Protocol

In September, more than 100 stakeholders from the Duchenne muscular dystrophy community gathered in Silver Spring, Md., to discuss the novel platform trial being proposed by I-ACT for Children, Parent Project Muscular Dystrophy and the Critical Path Institute. The goal of the platform trial is to speed the development of therapies for DMD by creating a platform through which multiple compounds can be tested at once. Potential benefits include:

- More rapid completion of testing and approval of new DMD therapies
- Reduced clinical trial start-up and execution time
- Enhanced patient experience, reduced number on placebo
- Potential for rapidly testing combination therapies

The meeting brought together researchers, Duchenne parents, advocacy groups, FDA representatives and drug sponsors to learn the details of the trial protocol and provide feedback on topics such as proposed eligibility criteria, endpoints, trial structure and governance. FDA’s Dr. Janet Woodcock, Director of the Center for Drug Evaluation and Research, and Dr. Billy Dunn, Director of CDER’s Division of Neurology Products, spoke at the start of the meeting about FDA’s interest in platform trials and their potential benefits for the DMD community. “We need to get work done and do it efficiently,” Dr. Dunn told the stakeholder audience. “That’s what this effort is all about.”

Links to the meeting summary and livestream of the meeting can be found here: https://www.iactc.org/dmd-stakeholder-meeting/

I-ACT for Children sees its role as a platform-trial incubator and is exploring the creation of such trials in other therapeutic areas.

Youth Tobacco-Cessation Strategies
Workshop a Success

In our last newsletter, we told of an upcoming FDA workshop we were hosting with the Duke Clinical Research Institute to understand what evidence exists to support youth tobacco-cessation efforts and where the treatment gaps lie. We are pleased to report that the workshop was a success, with nearly 900 people registered (in person and via webcast) to learn about existing experience with tobacco-cessation therapies in youth, the gaps in knowledge particularly relating
to the e-cigarette epidemic and where experts recommend putting future resources to fill those gaps.

Experts from academia, industry and the FDA shared their knowledge of the science of tobacco addiction in adolescents, the current state of behavioral and pharmacotherapy cessation strategies in adolescents and what is needed to generate robust evidence to address youth tobacco cessation. See https://www.iactc.org/youth-tobacco-cessation-meeting-material/ for a summary of the meeting, as well as the agenda and presentations.

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**Our Site Network is 52 Strong...**

The I-ACT Site Network now includes 52 sites with executed contracts, including multiple sites in Australia and one in Saudi Arabia. Our goal is to have 60 sites on board by the end of this year.

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... and continues to grow!
You can visit our 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.

Quality Improvement Pilot Launches at First 17 I-ACT for Children Sites

I-ACT for Children has launched the pilot phase of our quality improvement initiative at the first 17 sites in our network. This pilot phase of the Pediatric Improvement Collaborative for Clinical Trials & Research (PICTR®) program will assess the feasibility of collecting QI data across our broader site network.

In Q1 of 2020, we will work with Dr. Peter Margolis and his team at the James M. Anderson Center for Health Systems Excellence at Cincinnati Children's Hospital Medical Center to expand the program to 40 I-ACT for Children network sites. PICTR® will capture clinical trial operational data from each site that can be analyzed to determine where best practices are happening and where processes could improve. These data then will be shared and applied across the network to create a continuous learning environment designed to maximize trial speed, quality and efficiency.
1st Annual Pediatric Research Innovation Forum Addresses Inclusion of Adolescents in Adult Clinical Trials

I-ACT for Children hosted its 1st annual Pediatric Research Innovation Forum on Oct. 15-16, which focused on the inclusion of adolescents in adult clinical trials. The goal of the meeting was to drive innovation in this area by bringing key stakeholders together to agree on what’s known, identify continuing challenges and propose solutions to address those challenges.

There was robust engagement throughout the meeting and participation from multiple stakeholder groups – including leading scientific researchers, FDA representatives, patient advocates and industry research leaders. The meeting
drew 85 high-level participants from across the United States as well as from Spain, England, Canada and Germany.

I-ACT for Children now will work with meeting participants to develop a recommendations document for publication and dissemination, which will be informed by the Working Group sessions held during the meeting. Click here for the meeting presentations, photos from the event and the final meeting agenda: www.iactc.org/prif

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**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/](https://www.iactc.org/careers/)

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