

I-ACT for Children Site Network Newsletter January 2024



*Happy New Year to our fantastic Site Network Team!
Your collaborative spirit is what makes us strong.
Here's to achieving more together in the upcoming year.*

A YEAR IN REVIEW



2023 was a bustling year at FDA. The Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) gave the nod for 55 Novel drug approvals and 7 new cell and gene therapies in addition to dozens of supplemental and expanded label activities ([2023 Pediatric Drug Approvals](#)). It is notable that CDER identified 20 (36%) of the Novel drug approvals as being first-in-class, meaning they have a mechanism of action that differs from those of existing therapies.

With new approvals streaming out of FDA, we thought we would reflect over 2023 and share some of the pediatric highlights that caught our attention:

The **first-in-class** drug approval of **Daybue** provides an oral solution as the first treatment for patients aged 2 years and older with Rett Syndrome, a rare genetic neurological disorder that affects brain development.

A **new class of medicines** that are taken by mouth are now made available to treat pediatric type 2 diabetes, Sodium Glucose Cotransporter-2 (**SGLT2**) Inhibitors (**Jardiance** and **Synjardy**).

Vyjuvek was approved to treat wounds in patients with the dystrophic epidermolysis bullosa (DEB) with mutation(s) in the *collagen type VII alpha 1 chain (COL7A1)*. A genetically modified herpes-simplex virus type 1 (HSV-1) is used to deliver normal copies of the COL7A1 gene to wounds. COL7 molecules arrange themselves into long, thin bundles that form anchoring fibrils that hold the epidermis (skin) and dermis together, which is essential for maintaining the integrity of the skin.

Livmarli was approved to treat Cholestatic pruritus (extreme itching) in children 3 months and older with Alagille syndrome (ALGS). “The vast majority of patients are diagnosed with ALGS before one year of age. The availability of Livmarli will offer an opportunity to introduce a treatment at the beginning of their ALGS journey with the goal of reducing serum bile acids and alleviating the unrelenting burden caused by pruritus,” said Chris Peetz, president and chief executive officer at Mirum.”

Novel drug approved with the orphan drug designation. **Joenja** is a kinase inhibitor that corrects the immune defect in a genetic disorder that impairs the immune system (APDS). Joenja is approved as a First Treatment!

Accelerated approval was granted for **Rozlytrek**, a antineoplastic - Protein-Tyrosine Kinase Inhibitors (TKI) to treat solid tumors in children older than 1 month. The approval includes a new oral pellet formulation with instructions for making an oral suspension from the capsules.

FDA approves a CRISPR-based medicine for the treatment of sickle cell disease



The US Food and Drug Administration approved two gene-based treatments for sickle cell disease, including the first therapy that uses the gene-editing technique CRISPR. The essence of CRISPR is simple: it's a way of finding a specific bit of [DNA](#) inside a cell. After that, the next step in CRISPR gene editing is usually to alter that piece of DNA. However, CRISPR has also been adapted to do other things, such as turning genes on or off without altering their sequence.

These two gene-based treatments open a new era of treatments for genetic conditions. The medicines, called Casgevy and Lyfgenia, are potential cures for people with sickle cell, a debilitating and life-shortening inherited red blood cell disorder that disproportionately affects African Americans.

Casgevy is the CRISPR-based treatment made by Vertex Pharmaceuticals and CRISPR Therapeutics; Lyfgenia, made by Bluebird Bio, uses an older gene therapy approach. Both were cleared for people 12 and older with histories of vaso-occlusive crises and painful events caused by the disease.

ANNOUNCEMENTS

Quarterly Call
February 13, 2024 @ 12 pm ET
[Quarterly Call Registration](#)



Congratulations to our 2023 Champion of Excellence: Jurrans Wilson from Children's National Hospital.



Congratulations to our 2023 Educational Grant Awardee: Silvia Kwak from Northwell Health.

MENTORSHIP PROGRAM UPDATES

Thank You Mentors

3 Years
Amy Hanson
Jensina Ericksen
Jennie Wahl
Jurrans Wilson



2 Years

Angela Martino
Bonny Bowen
Elizabeth Hartigan
Jennifer Ward
Jessica Fritter

2 Years

Katie Mills
Misha Webb
Monica Bruton
Ping DeBlasio

Our Mentorship Program is now heading into our 4th year! What began as program of 22 participants has now grown in 3 years to 65 participants for 2024. We've made some exciting changes for this year by adding a collaboration tool in our Chatter function located within our Site Network Portal. This tool will give mentors and mentees a place to "chat" and collaborate, ask questions, and share tools and success stories. Another addition for this year's program is an optional monthly coffee hour that will be held via Zoom. This coffee hour will be a great opportunity for mentors and mentees to come together in real time to share thoughts, ideas and ask questions. For questions, feel free to contact the Site Network Team at SiteNetwork@iactc.org

SITE NETWORK PORTAL



Don't miss out on the Site Network Portal <https://iactc.my.site.com/sitenetwork/s/>

Portal Features

- ◆ Links to educational webinars
- ◆ News and Announcements
- ◆ Site Resources
- ◆ Communication/Collaboration Platform - Chatter Groups
- ◆ Templates - SOP, Job Ladders, Feasibility Checklists

EDUCATIONAL OPPORTUNITIES

2023 Advarra Study Activation Report

Advarra surveyed hundreds of clinical research professionals to understand the most pressing barriers potentially impacting study activation. Follow the link to read their report: [2023 Study Activation Survey Report](#)

FDA Podcast

Did you know that our Q&A with FDA Podcast now offers Continuing Education (CE) credit? Check it out now: <https://www.fda.gov/drugs/news-events-human-drugs/qa-fda>

Pediatric Video Series

The MRCT Center presented a five-part virtual conference series, Advancing International Pediatric Clinical Research, to advance the design, review, oversight, and conduct of global pediatric clinical trials. These videos, shared in the webinars, were produced in collaboration with the International Children's Advisory Network (iCAN.) <https://mrctcenter.org/resource/pediatric-video-series/>

UPCOMING CONFERENCES

SOCRA - Pediatric Clinical Trials Conference 2/21/24 - 2/23/24 - San Diego, California This conference provides attendees with new information, management tools, and real-life examples to help participants navigate the evolving landscape of pediatric research. [SOCRA Conference](#)

WCG MAGI - MAGI@home 10/16/23 - 10/20/23 - Virtual Clinical Research

The WCG MAGI Clinical Research Conference has remained an educational mainstay for clinical trial professionals for over 15 years. MAGI is going virtual this fall, bringing best-in-class, accredited training and education offerings from the comfort of your home or office. <https://www.wcgclinical.com/magi/>

ACRP- 2024 Conference 5/3/24 - 5/6/24 - Anaheim, California <https://acrpnet.org/event/acrp-2024>

YOUR SITE NETWORK TEAM



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