

**Best Pharmaceuticals for Children Act (BPCA)
Pediatric Oncology Working Group Conference Call
February 5, 2019
11:00 a.m.–11:15 a.m. (EST)**

Participants

Amy Barone, M.D.
Najat Bouchkouj, M.D.
Patricia Dinndorf, M.D.
Martha Donoghue, M.D.
Steven Dubois, M.D.
Ira Dunkel, M.D.
Lori Ehrlich, M.D.
Richard Gorlick, M.D.
Dionne Green, M.D.
Katherine Janeway, M.D.
Aviva Krauss, M.D.
Gregory Reaman, M.D.
C. Patrick Reynolds, M.D., Ph.D.
Nita Seibel, M.D.
Sonia Singh, M.D.
Malcolm Smith, M.D.
Perdita Taylor-Zapata, M.D.
Ashley Ward, M.D.
Brenda Weigel, M.D., M.Sc.
James Whitlock, M.D.
Joanna Yi, M.D.
Anne Zajicek, M.D., Pharm.D.

Purpose

The purpose of this call was to discuss the agenda for the upcoming June 2019 Pediatric Subcommittee of the Oncologic Drugs Advisory Committee (ODAC). During the 2-day meeting to be held at the U.S. Food and Drug Administration (FDA) White Oak campus, participants will focus on the following:

- Review of the potential applicability to the pediatric population of certain investigational products identified for discussion/presentation
- Whether meeting advisors recommended that Written Requests (WRs) should be issued to the sponsors of these products
- Two products currently being developed for graft versus host disease (GvHD).

June 2019 Pediatric Subcommittee of the ODAC Meeting: Draft Agenda

Dr. Reaman briefly explained that the upcoming Pediatric Subcommittee of the ODAC will be an opportunity for invited sponsors to present their products, including background and pre-clinical data, to discuss the potential applicability of these products in the pediatric population.

Dr. Reaman also explained that during this meeting, participants will be asked for their recommendations on whether or not to issue WRs to the sponsors developing these products. He briefly elaborated on the proposed agenda for the June 2019 meeting:

Day 1: Products for Discussion

- **Tegavivint:** Developer, Betacat Pharmaceuticals
- **ONC 201:** Developer, Oncoceutics, Inc.: This Akt/ERK inhibitor is being used in pediatric brain tumors. Dr. Barone explained that as a dopamine receptor inhibitor, this drug is also being studied in diffuse intrinsic pontine glioma (DIPG) and some adult brain tumors, as well as with the H3 K27M mutation.
- **KDO25:** Developer, Kadmon Corporation: This Rho-associated coiled-coil kinase 2 (ROCK2) inhibitor was originally developed for psoriasis and pulmonary fibrosis. The sponsor plans development of the product for treatment of chronic GvHD. Dr. Reaman pointed out that currently there are no approved products for chronic GvHD.
- **CD24Fc:** Developer, Oncolmmune, Inc.: This product is being developed as a preventive agent for GvHD.

Day 2: Review of Relevant Molecular Target Lists (1) Cell Lineage and (2) Immune Cells and Tumor Microenvironment

- Participants will have an opportunity to discuss what molecular targets should remain on the lists, as well as targets that might be removed, while others might be added.
- Additional presentations:
 - CAR-T cell opportunities in solid tumors, Presenter: Crystal Mackall
 - Novel cell membrane determinants for potential immunotherapy approaches to pediatric solid tumors, Presenter: John Maris

Other Products of Interest

Dr. Reaman asked WG members to continue to identify products that they would recommend adding to the June agenda or for presentation at future Pediatric Subcommittee ODAC meetings. He also noted that there has been some discussion about increasing the frequency of these meetings, while acknowledging the issues of limited time, especially given the legislatively mandated cumbersome approval process.

FDA Reauthorization Act (FDARA) Implementation: Impact of Partial Government Shutdown

Dr. Reaman briefly discussed the impact of the recent Government shutdown on FDA operations. He explained that this office continued to function, although there was a “hold” on accepting new applications. Dr. Reaman also noted that work on finalizing FDARA and Pediatric Research Equity Act (PREA) guidance was temporarily delayed. He assured participants that the draft FDARA implementation guidance is moving through clearance and will be ready to be published for review and comment.

Questions/Comments

Dr. Reynolds inquired about how to obtain the list of molecular targets. Dr. Reaman explained that the list is posted on the FDA website.

Dr. Weigel asked for additional information on the upcoming BPCA Annual Meeting. Dr. Taylor-Zapata noted that the BPCA meeting provides stakeholders an opportunity to review and discuss BPCA operations and programmatic initiatives in key areas, including supporting IND development and off-patent WRs focusing on pediatric oncology. Dr. Taylor-Zapata also encouraged WG participants interested in attending the upcoming BPCA meeting to contact her directly.

Next Scheduled Meeting

The next quarterly Working Group call is scheduled for Tuesday, May 7, 2019 at 11:00 AM Eastern Time.