Facilitating End-to-End Development of Individualized Therapeutics
Public Workshop – March 3, 2020
U.S. Food and Drug Administration
Center for Biologics Evaluation and Research (CBER)

Meeting Location:
FDA White Oak Campus
10903 New Hampshire Ave.
Bldg. 31 Conference Center Great Room
Silver Spring, MD 20993-0002

7:45 – 8:30 AM	Registration
8:30 AM	Introduction: **Dr. Gopa Raychaudhuri (CBER)**
8:35 AM	Speaker: **Dr. Peter Marks, CBER Center Director**
Session 1: MANUFACTURING	
8:55 AM	Session 1 Moderator Introduction: **Dr. Zenobia Taraporewala (CBER)**
9:05 AM	Speaker: **Dr. Guangping Gao, University of Massachusetts Medical School** *"Challenges and opportunities in development and manufacturing of individualized therapeutics with AAV vector-based gene therapies"*
9:25 AM	Speaker: **Dr. Jason Gill, Texas A&M University** *"Development of Bacteriophage Products"*
9:45 AM	Panel Session with Q & A [all speakers; Dr. Roger Plaut (CBER); Anita Richardson (CBER)]
10:15 – 10:30 AM	BREAK
Session 2: TOOLS FOR SAFETY TESTING AND DEVELOPMENT	
10:30 AM	Session 2 Moderator Introduction: **Dr. Sandhya Sanduja (CBER)**
10:40 AM	Speaker: **Dr. Albert Seymour, Homology Medicines, Inc.** *"Preclinical approaches/challenges in development of individualized therapeutics"*
11:00 AM	Speaker: **Dr. Malachi Griffith, Washington University School of Medicine** *"Bioinformatics tools for development, analysis, and preclinical testing of individualized therapeutics"*
11:20 AM	Speaker: **Dr. J. Keith Joung, Harvard Medical School and Massachusetts General Hospital** *"Preclinical testing platforms for genome editing"*
11:40 AM	Panel Session with Q & A [all speakers; Dr. Zuben Sauna (CBER)]
12:15 – 1:15 PM	LUNCH

Session 3: CLINICAL	
1:15 PM	Session 3 Moderator Introduction: **Dr. Rebecca Reindel (CBER)**
1:25 PM	Speaker: **Dr. Robert (Chip) Schooley, University of California, San Diego** *"Challenges and opportunities in the clinical development of bacteriophage"*
1:45 PM	Speaker: **Dr. Donald Kohn, University of California, Los Angeles** *"Challenges of developing academic-based clinical trials for pediatric diseases"*
2:05 PM	Panel Session with Q & A [all speakers; Dr. Larissa Lapteva (CBER); Dr. Zhenzhen Xu (CBER)]
2:35 – 2:50 PM	BREAK
Session 4: PRODUCTS TO PATIENTS	
2:50 PM	Session 4 Moderator Introduction: **Dr. Celia Witten, CBER Deputy Center Director**
3:00 PM	Speaker: **Ms. Jill Wood, Phoenix Nest Inc.** *"Role of stakeholders and collaborations needed for end-to-end development and sustainable delivery of biologics to treat diseases affecting one individual or a very small number of patients"*
3:20 PM	Speaker: **Dr. Alison Bateman-House, New York University Grossman School of Medicine** *"Ethical issues in product development and sustainability for individualized therapies"*
3:40 PM	Speaker: **Dr. Philip J. Brooks, National Institutes of Health** *"Beyond 'one disease at a time': Accelerating clinical trials of genetic therapies by grouping rare disease patients according to underlying disease mechanism"*
4:00 PM	Panel Session with Q & A [all speakers; Dr. Chip Schooley; Dr. Julie Vaillancourt (CBER)]
4:45 – 5:00 PM	Wrap up and Closing Remarks: **Dr. Peter Marks, CBER Center Director, FDA**