

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 <i>“Challenges and Opportunities in Development and Manufacturing of Individualized Therapeutics with AAV Vector-Based Gene Therapies”</i>
9:25 AM	Speaker: Dr. Jason J. Gill, Texas A&M University <i>“Development of Phage Therapy: Personalized Medicine and Individualized Therapeutics”</i>
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 B. Seymour, Homology Medicines, Inc. <i>“Preclinical Approaches/Challenges in Development of Individualized Therapeutics”</i>
11:00 AM	Speaker: Dr. Malachi Griffith, Washington University School of Medicine <i>“Bioinformatics Tools for Development, Analysis, and Preclinical Testing of Individualized Therapeutics”</i>
11:20 AM	Speaker: Dr. J. Keith Joung, Harvard Medical School and Massachusetts General Hospital <i>“Defining Off-Target Effects of Gene Editing Technologies”</i>
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 T. (Chip) Schooley, University of California, San Diego <i>"Opportunities and Challenges in the Clinical Development of Bacteriophage Therapeutics"</i>
1:45 PM	Speaker: Dr. Donald B. Kohn, University of California, Los Angeles <i>"Challenges to Developing Individualized Stem Cell Gene Therapies"</i>
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 A. Wood, Phoenix Nest Inc. <i>"The Trials and Tribulations of Driving a Treatment for an Uber-Rare Disease to the Clinic and Beyond - A Parent's Perspective"</i>
3:20 PM	Speaker: Dr. Alison Bateman-House, New York University Grossman School of Medicine <i>"Ethical Issues in Product Development and Sustainability for Individualized Therapies"</i>
3:40 PM	Speaker: Dr. Philip J. (P.J.) Brooks, National Institutes of Health <i>"Beyond 'one disease at a time': Accelerating Clinical Trials of Genetic Therapies by Grouping Rare Disease Patients According to Underlying Disease Mechanism"</i>
4:00 PM	Panel Session with Q & A [all speakers; Dr. Chip Schooley; CAPT Julie Vaillancourt (CBER)]
4:45 – 5:00 PM	Wrap up and Closing Remarks: Dr. Peter Marks, CBER Center Director, FDA