

CDER SMALL BUSINESS AND INDUSTRY ASSISTANCE



Oncology Therapy Development Workshop: Pivotal Steps and Avoiding Pitfalls for Start-ups

VIA WEBCAST www.fda.gov/CDERSBIA

MARCH 30-31, 2021

Version 9 – Updated March 26, 2021

For files and resources, please visit The Event Page on SBIAevents.com

Add Event to Your Calendar

AGENDA

All times are Eastern (EST UTC-4)

View Start Time on World Clock

DAY ONE: Tuesday, March 30, 2021

9:00 – 9:15

Administrative Overview

Brenda Stodart

CAPT, USPHS

Director, Small Business and Industry Assistance (SBIA)

Division of Drug Information (DDI) | Office of Communications (OCOMM) | CDER

9:15 – 9:20

Oncology Center of Excellence Introduction

Marc Theoret

Deputy Director

Oncology Center of Excellence (OCE)

Office of the Commissioner

9:20 – 9:35

Overview and Guide to the Workshop

Jeffrey Summers

Associate Director, Translational Sciences

Office of Oncologic Diseases (OOD) | CDER

9:35 – 10:30

Keynote: Innovation Mindset – Advancing Science to Therapies

Science is hard. With only a 1 in 10,000 likelihood of advancing a novel discovery to a commercial product, the process can be daunting, let alone difficult to navigate. Approaching each facet of this journey with an innovation mindset and clarity of purpose are key to improving both your odds of success and your experience (includes Q&A with presenter).

Keith Marmor

Chief Innovation &

Economic Engagement Officer

University of Utah

View Speaker Biographies

## DAY ONE: Tuesday, March 30, 2021

### Your SBIA Host for Day One

**Lisa Misevicz**  
*Health Communications Specialist*  
SBIA | DDI | OCOMM | CDER

10:30 – 11:00

### FDA Oncology Drug Development Overview - Past to Present

Provides a historical overview of oncology drug development past to present (includes Q&A with presenter).

**John Leighton**  
*Director*  
Division of Hematology, Oncology, Toxicology  
OOD | CDER

11:00 – 11:15

### Federal Resources for Innovative Cancer Startups: More than Just Funding!

In this talk, I will cover why SBIR funding is an attractive funding opportunity for startups, provide a primer on SBIR/STTR and discuss what makes a good SBIR application. I will discuss current funding opportunities, non-funding resources that can help accelerate commercialization and end with a few tips for a successful application.

**Deepa Narayanan**  
*Program Director & Team Lead*  
NCI Small Business Innovation Research  
Development Center  
National Cancer Institute, NIH

11:15 – 11:35

### Best Practices for Venture Capital Fundraising: Learn How Early-stage VCs Think

Two biotech venture capitalists will describe how investors think and what they are looking for in investment opportunities. They will also share tips for pitching to VCs and dos and don'ts of fundraising.

**Colleen Cuffaro**  
*Partner*  
Canaan Partners  
**Christy Shaffer**  
*Partner*  
Hatteras Venture Partners

11:35 – 11:50

### Funding Sources: Q&A Panel

**Deepa Narayanan, Colleen Cuffaro,  
Christy Shaffer**

**11:50 – 12:50: LUNCH BREAK**

**DAY ONE: Tuesday, March 30, 2021**

12:50 – 1:05

**Considerations for Building Your Broader Network and Value to Obtaining External Input Prior to Interacting with FDA**

As a former FDA reviewer, I will cover the limitations FDA has in providing detailed development strategy advice to programs and will share tips on getting the most out of your FDA interactions. With a view towards small companies with limited internal resources, I will share advice on how to broaden your advice network and what to look for in consultants and external partners to ensure product success.

**Julie Bullock**  
*Vice President*  
 Integrated Drug Development  
 Certara

1:05 – 1:20

**Assembling the Best Team to Navigate Through Preclinical Development**

This session will highlight some of the early-stage development challenges for startups, including some of the common pitfalls in planning and executing IND-enabling studies. We will focus on how to effectively transition from academic research to preclinical development and where to seek guidance along the way. Examples and tips for working with CROs will also be discussed.

**Christopher Scull**  
*Senior Consultant*  
 Biologics Consulting

1:20 – 1:35

**Consulting Companies and FDA Limitations: Q&A Panel**

**Julie Bullock, Christopher Scull**

1:35 – 2:05

**CMC Considerations for CAR T Cell Product Development**

This talk will outline the CMC requirements for CAR T cell product IND submissions. Specific aspects on early product development and product characterization will be discussed for both autologous and allogenic products (includes Q&A with presenter).

**Kimberly Schultz**  
*Gene Therapy Reviewer*  
 Division of Cellular & Gene Therapies  
 Office of Tissues and Advanced Therapies  
 (OTAT) | CBER

2:05 – 2:35

**CMC Considerations for Oncolytic Viral Product Development**

This talk will provide an overview of basic CMC requirements for oncolytic viral products. Specific topics will include recommendations on strategies and critical considerations for early phase product development, including control of the starting materials and reagents, the manufacturing process, and product testing (includes Q&A with presenter).

**Bo Liang**  
*Gene Therapy Reviewer*  
 Division of Cellular & Gene Therapies  
 OTAT | CBER

**2:35 – 2:45: BREAK**

## DAY ONE: Tuesday, March 30, 2021

2:45 – 3:35

### Preclinical Considerations for Cell and Gene Therapy Products, An FDA Perspective

In this talk, I will describe the preclinical program to inform early clinical development for cell and gene therapy (CGT) products. Particularly, I will provide considerations for relevant animal models and assessments for proof-of-concept, safety, and biodistribution to support first-in-human studies of CGT products in oncology, as well as updates of pathways for early communication with FDA/CBER (includes Q&A with presenters).

**Ying Huang**  
*Pharmacology/Toxicology Reviewer*  
Division of Clinical Evaluation and  
Pharmacology/Toxicology  
OTAT | CBER

3:35 – 4:15

### FDA's Clinical Regulatory Perspective: Designing First-In-Human Trial for Cellular and Gene Therapy Products

This session outlines key issues in reviewing first-in-human clinical protocols for cellular and gene therapy products for the treatment of cancer and describes pitfalls to avoid when designing these studies. Lessons learned from successful oncology cellular and gene therapy products and questions about clinical development will be addressed (includes Q&A with presenter).

**Peter Bross**  
*Chief (Acting)*  
Oncology Branch  
Division of Clinical Evaluation and  
Pharmacology/Toxicology  
OTAT | CBER

4:15 – 4:30

### Day One Closing

**Jeffrey Summers**  
*Associate Director, Translational Sciences*  
Office of Oncologic Diseases (OOD) | CDER

4:30: DAY ONE ADJOURN

## DAY TWO: Wednesday, March 31, 2021

9:00 – 9:05

### Overview from Your Day Two Host

**Lisa Misevicz**

*Health Communications Specialist*  
SBIA | DDI | OCOMM | CDER

9:05 – 9:10

### Welcome and Overview for Day Two

**Jeffrey Summers**

*Associate Director, Translational Sciences*  
Office of Oncologic Diseases (OOD) | CDER

9:10 – 10:10

### Chemistry and Manufacturing Requirements for Early Clinical Development: What's in there? Prove it.

CMC reviewers will provide insight into the FDA's perspective when reviewing early development IND submissions. An emphasis will be placed on common missteps that can lead to clinical holds for CMC deficiencies and critical documentation expected in the original IND submission (includes Q&A with presenters).

**Paresma Patel**

*Acting Branch Chief, Division of New Drug API*

**Olen Stephens**

*Chemist, Division of New Drugs Products 1*  
Office of Pharmaceutical Quality (OPQ)  
Office of New Drug Policy (ONDP) CDER

10:10 – 11:10

### CMC Considerations for Biotechnology Product Development: A Regulatory Perspective

This session provides an overview of regulatory expectations for biotechnology products, regulatory challenges, and strategies for success (includes Q&A with presenters).

**Wendy Weinberg**

*Chief, Laboratory of Molecular Oncology*

**Kristen Nickens**

*Product Quality Team Lead*  
Office of Biotechnology Products (OBP)  
Office of Pharmaceutical Quality (OPQ) | CDER

**DAY TWO: Wednesday, March 31, 2021**

11:10 – 11:40

**Getting to FIH for Small Molecules and Biologics**

This talk will cover the main guidance documents and nonclinical expectations for initiating trials and developing small molecule and biologic products for oncology drugs in CDER, as well as some common misconceptions and stumbling blocks (includes Q&A with presenter).

**Whitney Helms**  
*Supervisor, Pharmacology/Toxicology*  
 Division of Hematology, Oncology, Toxicology  
 OOD | CDER

11:40 – 12:20

**Designing First-in-Human Trials for Small Molecules and Biologics**

This presentation will provide an overview of the key design considerations for first-in-human trials of drugs intended to treat patients with cancer. Key topics include the approach to defining the patient population for eligibility, selection of the appropriate starting dose, dose escalation designs, protocol provisions to minimize patient risk, dose optimization, and initial assessment of antitumor activity for small molecules and biologics (includes Q&A with presenter).

**Martha Donoghue**  
*Acting Deputy Director*  
 Division of Oncology 2  
 OOD | CDER

**12:20 - 1:00: LUNCH BREAK**

1:00 – 1:55

**Planning for Co-development of Companion Diagnostics**

This talk will provide an overview of points to consider when planning for companion diagnostics including the use of investigational tests, how trial strategies can impact the companion diagnostic indication, common pitfalls to avoid, and how to obtain regulatory feedback (includes Q&A with presenter).

**Donna Roscoe**  
*Deputy Director*  
 Division of Molecular Genetics and Pathology  
 Office of In Vitro Diagnostics and Radiological Health  
 CDRH

1:55 – 2:40

**Clinical Development of Radiopharmaceuticals as Theranostic Pairs and Dosimetry Considerations for Therapeutic Radiopharmaceuticals**

Radiopharmaceuticals represent a growing portion of the armamentarium for treating cancer. In this context, theranostics refer to radiopharmaceutical therapies and nuclear medicine imaging drugs that share a mechanism of action and whose use may be coordinated during drug development, for pre-therapy dose and patient selection, and/or for post-therapy evaluation. This talk is focused on emerging pre-market pathways at FDA for theranostic co-development (includes Q&A with presenters).

**Anthony Fotenos**  
*Lead Medical Officer*  
**Donika Plyku**  
*Senior Staff Fellow*  
 Division of Imaging and Radiation Medicine  
 Office of Specialty Medicine  
 Office of New Drugs (OND) | CDER

**2:40 – 2:55: BREAK**

## DAY TWO: Wednesday, March 31, 2021

2:55 – 3:45

### Getting the Best Dose: The Clinical Pharmacology Studies that Help Achieve this Goal

The main goal of clinical pharmacology is to get the optimal dose for patients. I will discuss how the various clinical pharmacology studies help better understand dose optimization for different patients (includes Q&A with presenter).

**Brian Booth**

*Director, Division of Cancer Pharmacology 1  
Office of Clinical Pharmacology (OCP)  
Office of Translational Science (OTS) | CDER*

3:45 – 4:05

### Panel Discussion

**Jeffrey Summers**

*Associate Director, Translational Sciences  
Office of Oncologic Diseases (OOD) | CDER*

**Marc Theoret**

*Deputy Director  
Oncology Center for Excellence (OCE) | OC*

4:05 – 4:10

### Day Two Closing

**Jeffrey Summers**

*Associate Director, Translational Sciences  
Office of Oncologic Diseases (OOD) | CDER*

4:10: ADJOURN