Regulatory Strategy Forum for Biologics
Saturday, 9 September 2017

All attendees need to bring a fully charged laptop to the workshop. Limited power is available.

8:00 am	**Registration and Continental Breakfast**
9:00 am	**Introductions and Icebreakers** • This session will focus on introducing the panel members as well as introduction of participants to their assigned team. • Participants will be assigned to a team/table before they enter the conference room. **Linda Bowen, MS, RAC, FRAPS,** senior director, global regulatory science and policy, Sanofi **Eric Brass, MD, PhD,** professor emeritus of medicine, David Geffen School of Medicine at UCLA **Kamali Chance, MPH, PhD, RAC,** vice president and head, global biosimilars regulatory strategy, QuintilesIMS **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer **Ning Go, MD,** principal scientist, global IVD lead, clinical biomarkers and diagnostics, Amgen **Monica Siegenthaler, PhD,** regulatory affairs and quality assurance director, AiVita Biomedical **William Sietsema, PhD,** executive director, global regulatory affairs, Caladrius Biosciences
9:15 am	**Key Elements of Regulatory Strategy** This session will examine the basics of regulatory strategy formulation: • Why is it important to have a strategy? • What is involved in developing a strategy? • How is a strategy document typically structured? • What chapters does it contain? • Importance of assessing regulatory landscape, intended claims, speed to market • Considerations for country-specific and global regulatory strategy • How strategy development differs for biosimilars **Kamali Chance, MPH, PhD, RAC,** vice president and head, global biosimilars regulatory strategy, QuintilesIMS **William Sietsema, PhD,** executive director, global regulatory affairs, Caladrius Biosciences
9:45 am	**Introduction to Biologics Case Study** • This session will introduce participants to their fictional biologic, the global regulatory strategy template and the expectations for its completion. **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer

10:00 am	**Teams Discuss Their Biologics Case Study** • Teams will discuss amongst themselves about how to organize the work.
10:30 am	**Refreshment Break**
10:45 am	**The Regulatory Team** This session will evaluate: • The role of the regulatory team as strategic partners in business. • Discuss key players and obtaining buy-in for regulatory strategy. • How the team and its strategy may be impacted by a range of factors, such as business arrangements, regulatory environment changes, and the competitive landscape. **Monica Siegenthaler, PhD,** regulatory affairs and quality assurance director, AiVita Biomedical
11:15 am	**Companion Diagnostic Strategies** This session will cover: • Various codevelopment scenarios • Key codevelopment considerations and regulatory procedures in clinical investigation of a companion diagnostic **Ning Go, MD,** principal scientist, global IVD lead, clinical biomarkers and diagnostics, Amgen
11:45 am	**Working Lunch** • Teams may continue working on their case studies
12:45 pm	**Target Product Profiles** The Target Product Profile (TPP) is a living document that lays out the vision for the approval and optimal labeling of a safe and efficacious product. This session will: • Discuss the utility and value of a TPP • Provide an overview of the contents of a TPP • Advise on how to approach assembly of a TPP **Monica Siegenthaler, PhD,** regulatory affairs and quality assurance director, AiVita Biomedical
1:15 pm	**Clinical Development Strategies** This session will review key aspects of clinical development to enhance program efficiency and avoid challenges during regulatory review, including: • Maximize learnings from early clinical development • Optimize primary endpoint selection • Ensure adequacy of safety database **Eric Brass, MD, PhD,** professor emeritus of medicine, David Geffen School of Medicine at UCLA

1:45 pm	**Interchangeability of Biosimilars/Study Designs** FDA issued its long-awaited draft guidance for demonstrating biosimilar interchangeability in early 2017. This session will cover the following: • Demonstration of biosimilarity vs interchangeability • Interchangeability design considerations • Interchangeability as part of Phase 3 study design • Standalone interchangeability study design • Assessing interchangeability **Kamali Chance, MPH, PhD, RAC,** vice president and head, global biosimilars regulatory strategy, QuintilesIMS
2:15 pm	**Interacting with Regulators** Effective communications with regulators can help facilitate development and mitigate late stage regulatory challenges. Important skills include: • Formulating effective questions • Preparing briefing package • Creating a collaborative relationship • Elicit learnings from regulators' experience **Eric Brass, MD, PhD,** professor emeritus of medicine, David Geffen School of Medicine at UCLA
2:45 pm	**Refreshment Break**
3:00 pm	**Immunogenicity: Assessment and Implications for Regulatory Development** • Biological products can be recognized as foreign structures by the human immune system. High level concepts pertaining to immunogenicity assessment and the interpretation of regulatory relevance for different kinds of immunogenicity findings will be reviewed. **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer
3:30 pm	**Presentation of Team Biologic Case Studies** • In this session, groups will present their case study with an active Q&A session.
5:00 pm	**Adjourn**
6:00 pm	**Optional Networking Activity**

Sunday, 10 September 2017

7:00 am	**Registration and Continental Breakfast**
8:00 am	**FDA and EMA Thinking on Totality of Evidence in Development of Biosimilars** The regulatory landscape for the development of biosimilars in the US and EU is dynamic as many of the guidances issued by European Medicines Agency (EMA) and US Food and Drug Administration (FDA) have undergone revisions based on experience gained through review and approval of biosimilars for the respective regions. • Discover how biosimilars are regulated in the US and EU • Learn key elements of step-wise development program for biosimilars to meet global requirements • Learn key insights into interactions with the FDA regarding development of biosimilars • Learn key insights into interactions with EMA regarding development of biosimilars • Understand regulatory authority expectations for CMC, nonclinical and clinical similarity exercises **Kamali Chance, MPH, PhD, RAC,** vice president and head, global biosimilars regulatory strategy, QuintilesIMS
8:30 am	**The Preclinical/Early Clinical Interface for Biologics** • The high targeting specificity, including often species specificity, as well as the often extended half-life of biologics versus the typical small molecule, has led to somewhat different concepts being applied at the interface of preclinical and clinical development. Some considerations of regulatory concepts applying to early phase development of biologics, including the preclinical/clinical interface, will be reviewed. **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer
9:00 am	**Introduction to Biosimilars Case Study** • This session will introduce participants to their fictional biosimilar, the global regulatory strategy template and the expectations for its completion. **Kamali Chance, MPH, PhD, RAC,** vice president and head, global biosimilars regulatory strategy, QuintilesIMS
9:15 am	**Teams Discuss Their Biosimilars Case Study** • Teams will discuss amongst themselves about how to organize the work
9:45 am	**Refreshment Break**

10:00 am	**Regulatory Intelligence** • This session will provide a brief overview of the role of regulatory intelligence in developing a global regulatory strategy document. Presentation of various sources of information available to stay current on the global regulatory environment. Provide tips on how to search for different type of information. **Linda Bowen, MS, RAC, FRAPS,** senior director, global regulatory science and policy, Sanofi
10:30 am	**Biosimilars Case Study (continued)** • Teams continue discussion of the case study and preparing the presentation
11:00 am	**Comparability for Novel Biologics** • Biological therapies have compositional heterogeneity and this leads to an inability to demonstrate 'sameness' (as is typical for small molecules) which in turn has led to the development of the 'comparability paradigm.' Some very high-level concepts pertaining to comparability assessment for novel biologics will be reviewed. **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer
11:30 am	**Pediatric Strategy** • Europe and United States require either a pediatric plan or granting of a waiver. Europe urges a pediatric plan before conducting any pediatric studies and the United States expects a pediatric plan before conducting pivotal studies. Each region has its own procedures which might lead to separate studies by region, if no attempt is made to harmonize the studies. This presentation will review key regulations globally and offer suggestions for developing a globally harmonized and efficient pediatric plan. **William Sietsema, PhD,** executive director, global regulatory affairs, Caladrius Biosciences
12:00 pm	**Working Lunch** • Teams may continue working on their case studies
12:45 pm	**Global Regulation of Cell Therapies** • This session will provide an overview of how cell therapies are regulated in the United States, Europe, Canada, and Japan. **William Sietsema, PhD,** executive director, global regulatory affairs, Caladrius Biosciences

1:15 pm	**Orphan Strategies**
	• This session will discuss the strategic considerations in developing an orphan drug. Topics of discussion will include:
	  ○ Categories of orphan drugs and how they coincide with your regulatory strategy
	  ○ Regional differences and similarities in orphan drug programs
	  ○ Incentives, risks, and challenges to developing an orphan drug
	  ○ The impact and recent trends of orphan drug programs
	
	Monica Siegenthaler, PhD, regulatory affairs and quality assurance director, AiVita Biomedical

1:45 pm	**Advanced Biological Formats: Antibody-drug Conjugates, Bifunctionals, and Combinations: Special Regulatory Considerations** • There are currently numerous types of recombinant biologics in development and approved, but in order to engage a target with a toxic payload, or to engage multiple targets simultaneously, an increasing number of antibody-drug conjugates, bispecific molecules, and novel combinations of biologics are entering development. Special regulatory development considerations that apply to these modalities will be reviewed. **Vanessa D'Souza, PhD,** global regulatory team leader, Pfizer
2:15 pm	**Refreshment Break**
2:30 pm	**Presentation of Team Biosimilar Case Studies**
4:00 pm	**Adjourn**