TransCelerate BIOPHARMA INC.

Interpretation of Pharmacovigilance Regulations

Overview

Updated: 29 June 2017

Welcome to TransCelerate BioPharma Inc.

About TransCelerate

TransCelerate BioPharma Inc. was launched in 2012 as a non-profit organization with a mission to collaborate across the biopharmaceutical research and development community to identify, design and facilitate the implementation of solutions to drive efficient, effective and high-quality delivery of new medicines, improving the health of people around the world.

Our Mission Statement

TransCelerate BioPharma's mission is to collaborate across the global biopharmaceutical research and development community to identify, prioritize, design and facilitate implementation of solutions designed to drive the efficient, effective and high quality delivery of new medicines.

Our Members

TransCelerate BioPharma is comprised of the world's leading biopharmaceutical companies, with the vision of accelerating and enhancing the research and development of innovative new therapies. The spirit of innovation and collaboration occurs across TransCelerate Member Companies on Initiative goals.

Our Innovative Leaders

TransCelerate BioPharma is proud to have leadership from major biopharmaceutical companies – who have dedicated their careers to medicine, science and ensuring access to life-saving medicines through efficient and safe research and development.

We are fortunate to have the greatest minds in pharmaceutical R&D dedicated towards this common goal of identifying common issues and model solutions that will drive efficiencies into the R&D process.

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Pharmacovigilance Portfolio Vision

Future State

Patient-focused

Proactive

Evidence-based

Efficient

PV Guiding Principles

Sharpen focus *on the highest value safety information, increasing the signal to noise ratio*

Collaborate *to maximize learning and helping patients*

Build trust and confidence *with patients*

Share safety science *to allow for earlier identification of safety issues*

Explore use of modern technology *to increase value for patients and sponsors*

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Two Pharmacovigilance Initiatives

Interpretation of Pharmacovigilance Regulations

This initiative will share expertise more efficiently and effectively to meet the intent of pharmacovigilance requirements that seem ambiguous.

Value of Safety Information Data Sources

This initiative will identify sources of safety information for single high value valid cases and develop a proposed method for aggregate reporting of lower value cases.

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Interpretation of PV Regulations

UNMET NEED

Historically, there has been lack of a common understanding and approach regarding pharmacovigilance regulations. Specific challenges include:

- Disparate approaches to interpretation leading to misalignment in the industry
- Divergence in regulatory requirements across global health authorities

PROJECT DESCRIPTION

This initiative will share expertise more efficiently and effectively to meet the intent of pharmacovigilance regulations that seem ambiguous. The initial focus would be on the IND safety reporting regulations, with a future focus on operationalizing newly released regulations. An additional goal is to facilitate harmonization across regulators regarding regulatory expectations.

VALUE

Addressing these challenges will bring value to patients, sponsors, and regulators. Examples include:

- Improved patient safety through operational best practices for sponsors that meet the intent of pharmacovigilance regulations
- Reduced sponsor compliance risks through better understanding of regulations
- Harmonization across regulators through the proactive sharing of recommendations and best practices

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Approach

1 2 3 4 5

Repeat process for new regulation

Select regulation

- Evaluate potential regulations for interpretation based on pain points and timing of release
- Select appropriate team members to evaluate regulation

Evaluate regulation

- Identify ambiguous / pain point sections of regulation
- Determine underlying reasons for ambiguity / pain point

Develop position

- Determine best practices for operationalization
- Develop operationalization best practice position paper

Engage regulators

- Collaborate with and obtain feedback from health authorities on position

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Objectives

Near-term Objectives
2017

- Increase efficiency and efficacy across sponsors through the proposal of a reasonable interpretation of the IND safety reporting regulations
- Reduce inspection and compliance risk through a better understanding of the IND safety reporting regulations

Long-term Objectives
2018 and beyond

- Improve patient safety through development of operational best practices for sponsors that meet the intent of PV regulations
- Drive harmonization across health authorities by proactively sharing recommendations and best practices

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Expected Benefits

Near-term Benefits
2017

- Ability to **increase focus on proactive safety science** rather than interpretation of regulations
- **Reduced inspection and compliance risk** through a better understanding of the IND safety reporting regulations

Long-term Benefits
2018 and beyond

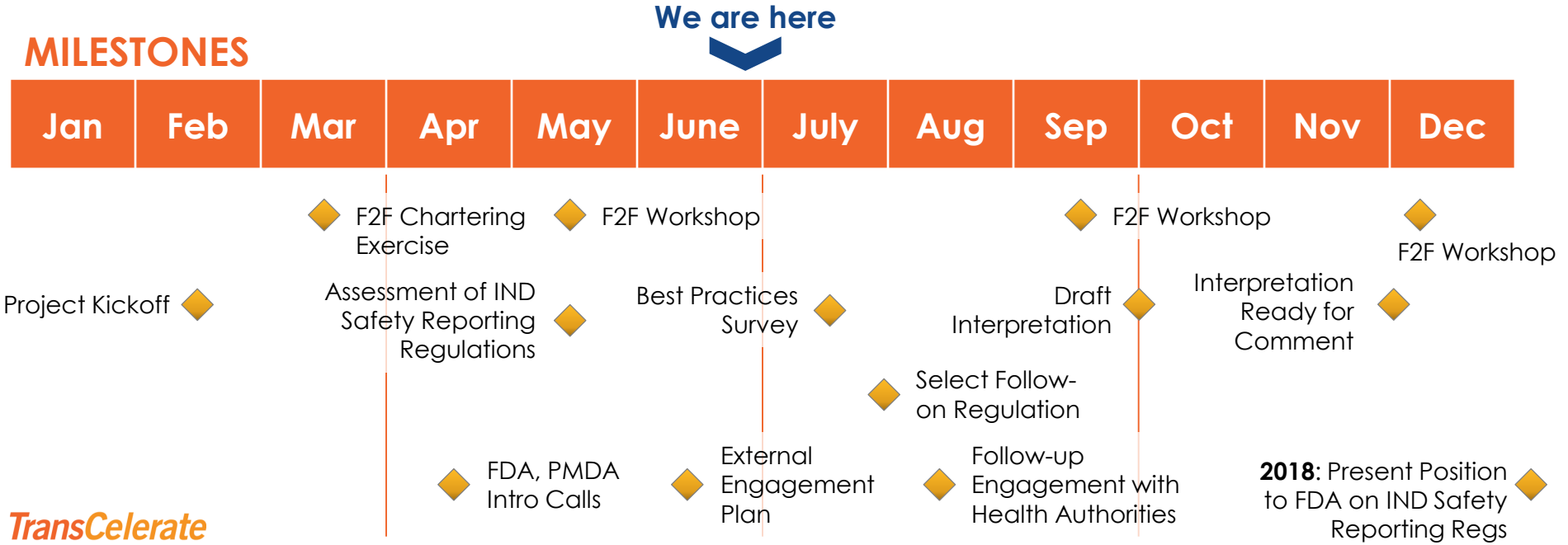
- **Improved patient safety** through operational best practices for sponsors that meet the intent of PV regulations
- **Harmonization across health authorities** through the proactive sharing of recommendations and best practices

Progress and Milestones

PROGRESS

- ✓ Assessed the FDA IND safety reporting regulations for ambiguous sections
- ✓ Drafted initial interpretation for key areas of the 2015 draft guidance to present to the FDA
- ✓ Determined mechanism for developing pipeline of additional regulations to interpret
- ✓ Created external landscape assessment and engagement plan
- ✓ Received positive feedback from initial meetings with FDA, PMDA, and EFPIA

MILESTONES



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Who's Involved

Sponsor	Jose Vega (Merck)
Workstream Lead	Robert Baker (Eli Lilly)

Core Team

abbvie
Allergan
AMGEN
AstraZeneca
Bristol-Myers Squibb
Boehringer Ingelheim
EMD SERONO MERCK
gsk GlaxoSmithKline
Johnson & Johnson
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THANK YOU

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