20 November 2018
EMA/493240/2018
Human Medicines Research and Development Support Division

Workshop with stakeholders on support to quality development in early access approaches (i.e. PRIME, Breakthrough Therapies)

26 November 2018, European Medicines Agency, London

Purpose

The European Medicines Agency (EMA) and the US FDA launched the PRIME and Breakthrough Therapy schemes to strengthen their support for the development of medicines that address unmet medical needs with the aim to help patients to benefit from these therapies as early as possible. Experience to date has shown that Applicants face challenges to complete quality and manufacturing development and data requirements during accelerated development. In order to address/overcome these challenges EU and US FDA Regulators wish to support Applicants with guidance and risk-based flexibility regarding their pharmaceutical development programme including, e.g. product characterisation, specification setting, validation and stability testing as well as early identification of quality issues / attributes that are critical to the clinical use of the medicinal product. The aim of this workshop, which constitutes a joint collaboration between EU regulators comprising BWP, QWP and IWG, and international partners including US FDA, is to discuss between Regulators and Industry these quality challenges and possible scientific and regulatory approaches which could be used to facilitate development and preparation of robust quality data packages, to enable timely access to medicines for patients whilst providing assurance that patient safety and product quality are not compromised.

These general discussions will be further elaborated through a number of specific industry case studies (covering chemical molecules, biologicals and ATMPs) and a discussion of experiences to date from early access approaches.

The conclusions from the workshop will be captured in a report, which will be published. The development of further follow-up guidance may be considered. The live broadcast can be followed on the link below under Multimedia tab. https://www.ema.europa.eu/events/workshop-stakeholders-support-quality-development-early-access-approaches-ie-prime-breakthrough

Location

European Medicines Agency
30 Churchill Place, Canary Wharf
London E14 5EU
United Kingdom

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Agenda

26 November 2018, 09:00-17:30 GMT

Chairperson: S. Ruiz (BWP chair, EU)

Timings	Topic
8.30-9.00	Arrival and registration
9.00-9.05	**Welcome & Introductions** *E. Alteri (Head of Human Medicines Research and Development Support Division, EMA)*
1. Background & Scope of early access approaches *Session leads: V. Jekerle (Quality Office, EMA) and R. Sood (CDER, FDA)*	
9:05-9:15	**Goals of Workshop & problem statement** *S. Ruiz (BWP chair, EU)* *K. Pugh (QWP chair, EU)* *V. Jekerle (Quality Office, EMA)*
9:15-9:25	**Perspective from US-FDA** *R. Sood (CDER, FDA)*
9:25-9:35	**Perspective from EU-EMA** *V. Jekerle (Quality Office, EMA)*
9:35-9:45	**Industry case study** In-licensed small molecule oncology drug: challenges/complexity of accelerated development with multi-Health Authority interactions. *F. Schwarb (Roche)*
2. Process validation *Session lead: S. Barry (BWP member, EU)*	
9:45-9:55	**Regulator's perspective** *S. Barry (BWP member, EU)*
9:55-10:10	**Industry case studies** Innovative validation. *S. Finnie (Astra Zeneca)* Process Validation Approaches for Accelerated Programs. *L. de Cardenas (Genentech)*
10:10-10:30	**Panel discussion** *Regulators: S. Barry (BWP member, EU), J. Limberg (QWP member, EU), E. Lacana and M. Ramanadham (CDER, FDA)* *Industry: S. Finnie (Astra Zeneca) and L. de Cardenas (Genentech)*

Timings	Topic
	Coffee break 10:30 – 10:45
3. Control strategy *Session lead: M. Welin (BWP member, EU)*	
10:45-11:05	**Regulator's perspective** *M. Welin (BWP member, EU)* *L. Graham (CDER, FDA)*
11:05-11:25	**Industry case studies** Use of Prior Knowledge to Establish Flexible Enhanced Model-based Control Strategies. *D. Wilkinson (Biogen)* CMC information to support Vaccine Early Access designation- Composite Case Study from Vaccine Manufacturers (GSK, Janssen, MSD, Pfizer, Takeda). *C. Campa (GSK)*
11:25-12:00	**Panel discussion** *Regulators: M. Welin and N. Kruse (BWP members, EU), T. Agasoster (QWP member, EU), L. Graham (CDER, FDA)* *Industry: D. Wilkinson (Biogen) and C. Campa (GSK)*
4. GMP-compliance *Session lead: G. Lorenti (IWG member, EU)*	
12:00-12:05	**Regulator's perspective** *G. Lorenti (IWG member, EU)*
12:05-12:15	**Industry's case study** Perspective on GMP Considerations for Accelerated Access. *M. Popkin (GSK)*
12:15-12:30	**Panel discussion** *Regulators: G. Lorenti (IWG member, EU), M. Ramanadham and L. Graham (CDER, FDA)* *Industry: M. Popkin (GSK), M. Ganapathy (MSD), A. Lodge (Kite Pharma)*
	Lunch break 12:30 – 13:30

Afternoon parallel sessions 13:30 – 15:00

Biologicals, room 2A Session chair: S. Ruiz	
5a. Case studies on process validation and control strategy *Session lead: S. Barry (BWP member, EU)*	
13:30-13:50	**Industry case studies** Approaches to Setting Specification Acceptance Criteria. *R. Keane (Biogen)* Ebola case study on process validation and control strategy. *T. Pepper (MSD)*
13:50-14:00	**Panel discussion** *Regulators: S. Barry (BWP member, EU), E. Lacana and L. Graham (CDER, FDA) and A. Byrnes (CBER, FDA)* *Industry: R. Keane (Biogen), T. Pepper (MSD), A. Lennard (Amgen)*
6a. Comparability *Session lead: M. Hoefnagel (BWP member, EU)*	
14:00-14:30	**Industry case studies** Risk-based assessment of comparability for a mAb *A. Clinch (UCB)* ATMP comparability challenge case study. *M. Jeschke (Novartis)* Approaches to Comparability. *M. Alai-Safar (Kite Pharma)*
14:30-14:40	**Panel discussion** *Regulators: M. Hoefnagel (BWP member, EU), E. Lacana (CDER, FDA) and A. Byrnes (CBER, FDA)* *Industry: A. Clinch (UCB), M. Jeschke (Novartis), M. Alai-Safar (Kite Pharma)*
7a. Stability *Session lead: M. Welin (BWP member, EU)*	
14:40-14:50	**Industry case study** Stability: Predictive Stability Models to Extrapolate Shelf-life. *A. Lennard (Amgen)*
14:50-15:00	**Panel discussion** *Regulators: M. Welin (BWP member, EU), L. Graham and E. Lacana (CDER, FDA), A. Byrnes (CBER, FDA)* *Industry: A. Lennard (Amgen), M. Goese (Roche)*
Coffee break 15:00 -15:30	

Chemicals, room 2D Session chair: K. Pugh	
5b. Control strategy *Session lead: K. Olofsson (QWP member, EU)*	
13:30-14:00	**Industry case study** Impurity Control Strategy for an Oncology drug. *A. Teasdale (Astra Zeneca)*
14:00-14:15	**Panel discussion** *Regulators: K. Olofsson (QWP member, EU), M. Ramanadham, S. Furness and R. Sood (CDER, FDA)* *Industry: A. Teasdale (Astra Zeneca)*
6b. Stability *Session lead: T. Agasoster (QWP member, EU)*	
14:15-14:40	**Case studies from industry** Supporting Accelerated Development - STABILITY approaches. *R. Ogilvie (Pfizer)*
14:40-15:00	**Panel discussion** *Regulators: T. Agasoster (QWP member, EU), S. Furness and R. Sood (CDER, FDA)* *Industry: R. Ogilvie (Pfizer), A. Kuzmission (Vertex), M. Ganapathy (MSD)*
Coffee break 15:00 -15:30	

Afternoon joint session

Timings	Topic
8. Summary of the BIO and CHE afternoon discussions	
15:30-15:45	**Summary of the afternoon sessions** *M. Hoefnagel (BWP member, EU) and J. Limberg (QWP member, EU)*
9. Regulatory tools to support early access *Session lead: D. Hernan (Quality Office, EMA)*	
15:45-16:00	**Industry perspective & case study** Regulatory tools to support early access – Industry perspective. *Y. Momonoi (Celgene)*
16:00-16:20	**Regulators' presentations on regulatory tools** *C. Blanc (Procedure Management, EMA) and C. Bouygues (RA Office, EMA)*

Timings	Topic
	A. Byrnes (CBER, FDA)
16:20-17:00	**Panel discussion** *Regulators: C. Blanc (Procedure Management, EMA), C. Bouygues (RA Office, EMA), K. Olofsson (QWP member, EU), M. Hoefnagel, (BWP member, EU), A. Byrnes (CBER, FDA), M. Ramanadham and E. Lacana (CDER, FDA)* *Industry: Y. Momonoi (Celgene), D. Wilkinson (Biogen), M. Goese (Roche)*
10. General discussion, summing up and way forward	
17:00-17:30	**Lessons learned from the workshop and next steps** *S. Ruiz (BWP chair, EU)* *K. Pugh (QWP chair, EU)* *L. Graham (CDER, FDA)* *A. Hidalgo Simon (Head of Specialised Scientific Disciplines Department, EMA)*
End of meeting 17:30	

Workshop organising committee

EU

Giampiero Lorenti, Agenzia Italiana del Farmaco, Italy, IWG member

Jobst Limberg, Bundesinstitut für Arzneimittel und Medizinprodukte, Germany, QWP member

Kristofer Olofsson, Läkemedelsverket, Sweden, QWP member

Marcel Hoefnagel, College ter Beoordeling van Geneesmiddelen, The Netherlands, BWP expert

Mats Welin, Läkemedelsverket, Sweden, BWP member

Sean Barry, An tÚdarás Rialála Táirgí Sláinte, Ireland, BWP member

Tone Agasoster, Statens legemiddelverk, Norway, QWP member

FDA

Andrew Byrnes, Office of Tissues and Advanced Therapies, CBER

Emanuela Lacana, Office of Biotechnology, OPQ, CDER

Judith Arcidiacono, Office of Tissues and Advanced Therapies, CBER

Laurie Graham, Office of Policy for Pharmaceutical Quality, OPQ, CDER

Mahesh Ramanadham, Office of Process and Facilities, CDER

Ramesh Sood, Office of New Drug Products, OPQ, CDER

Scott Furness, Office of New Drug Products, OPQ, CDER

EMA

Veronika Jekerle, Quality Office, European Medicines Agency

Dolores Hernan, Quality Office, European Medicines Agency

Kaidi Koiv, Quality Office, European Medicines Agency

Speakers & Panellists

Regulators

Sol Ruiz, Agencia Espanola de Medicamentos y Productos Sanitarios, BWP chair

Keith Pugh, Medicines and Healthcare Products Regulatory Agency, QWP chair

Enrica Alteri, Head of Human Medicines Research and Development Support Division, EMA

Ana Hidalgo Simon, Head of Specialised Scientific Disciplines Department, EMA

Peter Richardson, Head of Quality Office, EMA

Caroline Blanc, Procedure Management, European Medicines Agency

Christelle Bouygues, Regulatory Affairs Office, European Medicines Agency

Industry

Alexandra Clinch, UCB

Andrew Kuzmission, Vertex

Andrew Lennard, Amgen

Andrew Teasdale, Astra Zeneca

Anthony Lodge, Kite Pharma

Cristiana Campa, GSK

Diane Wilkinson, Biogen

Fabian Schwarb, Roche

Lisa de Cardenas, Genentech

Margit Jeschke, Novartis

Markus Goese, Roche

Matt Popkin, GSK

Mehrshid Alai-Safar, Kite Pharma

Mohan Ganapathy, MSD

Richard Keane, Biogen

Ron Ogilvie, Pfizer

Stuart Finnie, Astra Zeneca

Teresa Pepper, MSD

Yoko Momonoi, Celgene