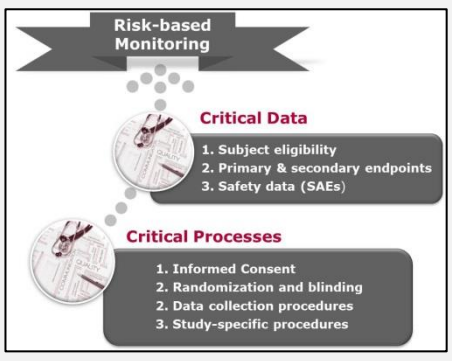
Newsletter

January 2018 – Innovation on the Rise

Photography provided by Dr. Dorothé Eisermann

A New Year Driven by Innovation and Globalization

"Innovation distinguishes between a leader and a follower." - Steve Jobs



Drug developers look beyond the horizon to increase medicines effectiveness and patient access to highly needed therapies, improved by patient-centered tactics.

We invite change embracers to visit All about RBM, a repository of the most relevant guidelines and available tools (e.g., RACT plus) concerning risk-based Quality Management under the PPH plus Knowledge Center section of our website.

EMA on Multi-regional Clinical Trials

The EMA has approved the ICH guideline E17 on general principles for planning and design of multi-regional clinical trials (MRCTs), which will come into effect on 14 June 2018.

The new guideline aims at increasing the suitability of multiregional clinical trial designs for global regulatory submissions, addressing some strategic program as well as specific planning and design aspects of confirmatory MRCTs. This document complements the ICH efficacy guidelines E2, E3, E4, E5, E6, E8, E9, E10 and E18.

EMA on Orphan Medicines

The European Medicines Agency (EMA) has published a new fact sheet on the EU's orphan designation program (launched in the year 2000) and the incentives made available to developers therein.

According to the EMA, there are over 6,000 rare diseases. 140 out of over 1,900 orphan-designated medicines reached the market by the end of 2017. These orphan medicines are aimed at fulfilling the needs of around the estimated 30 million patients with rare diseases (1 in 17 people) in the EU who, otherwise, will have no treatment. A sponsor's guide to an orphan designation and further regulatory guidance are available on the EMA website.

FDA on Targeted Therapies

Instead of focusing on a specific disease, targeted therapies tackle specific disease features. Giving the rising development of targeted therapies, two new guidelines have been released by the FDA. The first one provides guidance for industry on developing targeted therapies in low-frequency molecular subsets of a disease. This guideline addresses how to enroll patients based on the identification of rare genetic mutations into clinical trials for targeted therapies, comprising "tissue agnostic" drug development.

In the development of new, effective and safe targeted therapies, investigational in vitro diagnostics (IVDs) (i.e., to assess biomarkers and select therapies) are often used in clinical trials. Thus, the complementary FDA guideline focusses on investigational IVDs used in clinical investigations of therapeutic products. This document also guides sponsors on when an in vitro diagnostic (IVD) device used must undergo its own FDA review.

FDA on Medical Devices

In order to encourage digital innovation, the FDA issued two new draft guidelines to change medical software policies and one final guideline on the clinical evaluation of software as a medical device (SaMD) on 8 December 2017.

On the other hand, the FDA states (press announcement) to have reviewed over 100 devices currently on the market that were manufactured on 3D printers (e.g., implants for facial reconstruction). The new FDA guideline on technical considerations for additive manufactured medical devices (MD) guides manufacturers on technical aspects of 3D printing and on what to include on submissions for 3D-printed MDs.

***Save the Date:** **20 April 2018** - PPH plus XIII Annual Interdisciplinary Drug Development Expert Workshop, Frankfurt am Main, Germany.

How can we help you?

2016 | 2017 INNOVATIVE THROUGH RESEARCH Awarded by the Stifterverband PPH2594FRA

Awarded to PPH plus 05/2016

CONTACT

Dr. med. Johanna Schenk

Managing Director &

Chief Operating Officer

johanna.schenk@pph-plus.com

Phone +49 (0)69 587 00 35 10

Dr. med. Peter Klöpel

Director Consulting Services

peter.kloepel@pph-plus.com

Phone +49 (0)69 587 00 35 40

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PPH plus GmbH & Co. KG

Altenhöferallee 3

60438 Frankfurt am Main

Germany

www.pph-plus.com