PVRI

Innovative Drug Development Initiative (IDDI) Report

2020

Mission accomplished or more to come?

Report by Paul Corris, Sylvia Nikkho & Peter Fernandes

The Innovative Drug Develpoment Initiative (IDDI) was established in 2017 under the leadership of Paul Corris (PVRI Chair of the Board), Sylvia Nikkho (Bayer) and Peter Fernandes (Bellerophon Therapeutics). The IDDI is a unique core activity within the PVRI facilitating the development of novel treatments for patients with pulmonary vascular diseases (PVD) and establish them into clinical practice.

IDDI provides a platform for academia, the pharmaceutical industry, patient representatives and drug regulators to openly discuss questions surrounding the future of trials comprising novel drugs. Importantly, the four workstreams: clinical trial design; endpoints; biomarkers; and repurposed drugs were interlinked and mutually supportive and chaired by an expert from academia and one from pharmaceutical industry.

In 2020 the IDDI completed its major goal to establish a series of guidance documents in *Pulmonary Circulation*:

1. **Clinical trial design in phase 2 and 3 trials for pulmonary hypertension. October 2020.** READ HERE
2. **Novel composite clinical endpoints and risk scores used in clinical trials in pulmonary arterial hypertension. October 2020.** READ HERE
3. **Role of biomarkers in evaluation, treatment and clinical studies of pulmonary arterial hypertension. October 2020.** READ HERE
4. **Repurposing of medications for pulmonary arterial hypertension. October 2020.** READ HERE

As noted in the editorial, this collection of leading experts reflect on previous experience, review present understanding, and consider how their respective areas of expertise may develop in the years ahead.

There is more to come, PVD matters to patients. It is still a field of high unmet medical need. Although great progress has been made in Pulmonary Arterial Hypertension and Chronic Thromboembolic pulmonary hypertension with several treatment options available, there is still no cure for those indications and no disease specific treatment for other forms of PH. Studies are getting more and more complex with endpoints not seen as meaningful *(6-miniute walking distance)* or feasible *(time to clinical worsening)* anymore. Ideas on novel clinical trial design and endpoints require regulatory

The IDDI has expanded.

acceptance based on evaluation of existing registory or clinical data using artificial intelligance. Furthermore, PVD is not only a disease in developed contries, it also needs to be recognised as a global burden with access to medicine ensured.

The IDDI has therefore been expanded to **IDDI Global Alliance** co-chaired by **Raymond Benza** (Ohio State University, Columbus, USA), **Mark Toshner** (Papworth Hospital, Cambridge, UK), **Sylvia Nikkho** (Bayer, Berlin, Germany) and **Peter Fernandes** (Bellerophon Therapeutics, New Jersey, USA).

Its mission is to get PVD recognised as a global burden of disease, working in collaboration with international health organisations on "classical" IDDI topics. These include the challenges of clinical trial design and endpoints, new modalities and technologies, real world evidence, patient engagement, specific features of PH groups, patient engagement and last but not least, Covid-19's impact on PH patients and PVD long-term sequalae.

The Emerging Biotechnology Consortium (EBC), was initiated in late 2020 under the leadership of Peter Fernandes and Ray Benza, creating work streams to focus on the development of newer technologies (covering both diagnostics and devices) that provide a therapeutic benefit, patient monitoring/care or novel inhalation drug delivery roles. Each work stream will provide EBC members a much needed platform within PVRI and IDDI where the voice of smaller emerging biotechnologies will be heard and acted upon. The EBC is integrated formally within the broader umbrella of the IDDI/Global alliance.

A note of thanks

On behalf of the PVRI, we would like to express our congratulations to Sylvia Nikkho for her induction into Bayer's Science Fellow Programme as a Senior Science Fellow. This is a fantastic and well-deserved achievement.

We would like to express our sincere thanks to the dedicated leaders and members of all the IDDI work streams and in particular our PVRI Roundtable Members. ■