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RESEARCH & INNOVATION FOR NEGLECTED DISEASES

KEY RECOMMENDATIONS

from product development
partnerships (PDPs) for

Inclusion in Horizon 2020



OPPORTUNITIES:

How can the EU help fill the research gap for poverty-related and neglected diseases?

IMPROVED MECHANISM

- Funding should support the efficient management and linkage of basic research, translational research, clinical trials, launch, and implementation to facilitate access to health technologies
- Funding mechanisms should consider innovative partnerships models outside of classic consortia and project funding, ensuring that it supports fast evolving product pipelines.

ADDITIONAL FUNDING

- Substantial increase of funding for research and development for poverty-related and neglected diseases in HORIZON 2020.
- Ensure that long-term, flexible funding for global health product development is available.

EUROPEAN AND DEVELOPING COUNTRIES CLINICAL TRIALS PARTNERSHIPS (EDCTP-II)

- Ensure that EDCTP-II will be realized with an increased budget, flexible funding criteria, extension to neglected tropical diseases, and clinical trial Phases 1 through 4, as well as extended to include other developing countries.

WHAT ARE PDPs?

PDPs bring together contributions—financial, technical and in-kind—from the public sector, private sector, nongovernmental organizations (NGOs) and academia to further the development of new health technologies targeted to the needs of developing countries.

Through this innovative mechanism, PDPs are able to advance global health goals by accelerating the development of products that may not otherwise have been created.

- PDPs are public health-driven.
- PDPs are nonprofit organizations.
- PDPs work in partnership with academic institutions, governments, and the private sector.
- PDPs focus on one or more poverty-related and neglected diseases and develop health innovations that are suitable and affordable to ensure access and use in developing countries.
- PDPs cover the full innovation cycle, from early discovery stages to the implementation of a product.
- PDPs typically use private sector management practices to drive product development.

PDPs are a proven model:

Prior to the creation of PDPs, the neglected disease research and development (R&D) pipelines were noticeably empty; one study estimated there were only 20 development projects for neglected disease drugs between 1975 and 2000¹, and the historic lack of investment resulted in only 18 of the 1,535 medicines having indications for poverty-related and neglected diseases, accounting for 41 percent of the 1.5 billion disability adjusted life years.² This is a global concern.

Since their establishment, PDPs have re-catalysed the development of global health tools. To date PDPs have developed and licensed 19 products for use in the developing world; in 2009, PDPs had nearly 150 biopharmaceutical, diagnostic, and vector control candidates in various stages of development, including 32 in late-stage clinical trials. Paradoxically, just when many PDPs are on the cusp of achieving

significant results, public funding for product development dropped markedly.³

PDPs have helped to create the largest global health product development pipeline ever for drugs, vaccines, and diagnostic tools. In total, PDPs are planning more than 100 active and new clinical studies in 2011-2012. They plan to conduct 142 total studies in 45 countries in 20 disease areas.



¹ Morgan MA. New EU approaches to funding R&D for neglected diseases. Pharmaceutical R&D Policy Project. March 2005; London, England.

² Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions., HORIZON 2020 ,The Framework Programme, November 11, 2011.

³ Moran M, Guzman J, Abela-Oversteegen L, et al. Neglected disease research and development: Is innovation under threat? G-Finder 2011. Policy Cures; 2011. Available at: http://www.policycures.org/downloads/g-finder_2011.pdf.

HOW DO PDPs FIT INTO THE EUROPE 2020 STRATEGY?

PDPs leverage private and public engagement.

By leveraging public and private-sector resources PDPs reduce the cost of development for new health innovations. They engage effective partners to accelerate the process of scientific discovery to serve patients' needs. By investing in PDPs, public donors reduce risks and save tax-payers money as PDPs leverage investments from industry partners.

PDPs attract pharmaceutical companies and biotech firms that have relevant expertise and knowledge, but have not been working on poverty-related and

neglected diseases before. PDPs have helped identify promising products, brought together partners working on similar projects, created synergies between projects and partners, helped create a larger and more stable market, and provided other incentives including seed funding and project management. The research has also provided new opportunities for academia to work with industry so they can see their projects moving quickly from the lab to clinical development and to patients. Importantly, PDPs also actively engage research

partners in developing countries, stimulating sustainable medical research in these endemic countries and linking scientists and scientific efforts across the geographic North-South divide.

In Europe PDPs engage with many research institutions and pharmaceutical companies. In doing so, PDPs stimulate and contribute to the robust European research and development sector and facilitate access to work globally in international research partnerships.

PDPs WORK WITH A VARIETY OF EUROPEAN WORLD-CLASS RESEARCH INSTITUTIONS AND PRIVATE SECTOR PARTNERS.

Some examples are:

- Academic Medical Center (the Netherlands)
- AlgoNomics (UK)
- AstraZeneca UK Limited (UK)
- Bayer AG (Germany)
- Biotica Technology Ltd (UK)
- Biovex (UK)
- Centre de Recerca en Salut Internacional de Barcelona (CRESIB) (Spain)
- Centre National de la Recherche Scientifique (France)
- Complix MV (Belgium)
- Cranfield University (UK)
- Crucell N.V. (Netherlands)
- Deutsches Medikamentenhilfswerk action medeor e.V., Tonisvorst (Germany)
- F. Hoffmann-la Roche Ltd. (Switzerland)
- Ferrer Research and Development (Spain)
- GlaxoSmithKline (UK)
- GlaxoSmithKline Biologicals (Belgium)
- European Molecular Biology Laboratory (Germany)
- Imperial College of Science, Technology and Medicine (UK)
- Intercell (Austria)
- Institute for Molecular Biology and Biotechnology, Brussels
- Institute Pierre et Marie Curie (France)
- Institute Pasteur (France)
- Karolinska Institute (Sweden)
- Lipoxen (UK)
- London School of Tropical Hygiene and Medicine (UK)
- Novartis Pharma AG (Switzerland)
- Pepsican Therapeutics (Netherlands)
- Pfizer Limited (UK)
- Sanofi (France)
- Skau Vaccines (Denmark)
- Sigma-Tau Industrie Farmaceutiche Riunite S.A (Italy)
- Statens Serum Institut (Denmark)
- Swiss Tropical and Public Health Institute (Switzerland)
- University of Antwerp (Belgium)
- University of Bristol (UK)
- Universiteit Brussel (Belgium)
- Université de la Méditerranée (France)
- University of Dundee (UK)
- University of Galway (Ireland)
- University of Liverpool (UK)
- University of Oxford (UK)
- University of Regensburg (Germany)
- University of Strasbourg (France)
- Universität Tübingen (Germany)
- Vrije (Netherlands)
- Xenetic Biosciences (UK)

19 PRODUCTS PRODUCED BY PDPs

include tools to combat malaria, sleeping sickness, cholera, Japanese encephalitis, meningitis, VL and tuberculosis in low- and middle- income countries.

1. ASAQ (artesunate/amodiaquine) (DNDi)
2. ASMQ (artesunate/mefloquine) (DNDi)
3. NECT (Nifurtimox Eflornithine Combination Therapy) (DNDi)
4. SSG&PM Combination Therapy in Africa (DNDi)
5. Xpert MTB/RIF (or automated nucleic acid amplification) (FIND)
6. Liquid culture (FIND)
7. Rapid speciation for MDR-TB (FIND)
8. LPA line probe assay (FIND)
9. Fluorescence microscopy (FIND)
10. KalazarDetect (IDRI)
11. Paromomycin (OWH)
12. Killed whole-cell oral cholera vaccine (IVI)
13. Coartem® *Dispersible* (MMV with Novartis)
14. Injectable artesunate (MMV with Guilin Pharmaceuticals)
15. Eurartesim® (MMV with Sigma-Tau)
16. Pyramax® (MMV with Shin Poong)
17. MenAfriVac (MVP)
18. JE Vaccine India (PATH)
19. LAPEPE Paediatric dosage form of benznidazole (DNDi)

RECOMMENDATIONS FOR THE FRAMEWORK PROGRAMME FOR RESEARCH AND INNOVATION

The process of researching, developing, and delivering life-saving health products for neglected diseases for use in developing countries could be accelerated through specific changes in the HORIZON 2020:

- Substantial increase of funding for research and development into neglected and poverty-related diseases. Currently, only four percent of the overall European Commission (EC) contribution to health R&D under FP7, or €250 million, is dedicated to research for AIDS, tuberculosis, and malaria. Even less is available for other neglected diseases.
- In order to achieve a greater impact, funding should support the efficient management and linkage of basic research, translational research, clinical trials, access activities, and implementation of health products, underpinned by solid access commitments throughout the R&D process, including appropriate agreements on intellectual property. The EC should ensure that funding is available to organizations with the ability to address the full innovation cycle, such as PDPs.
- Current funding mechanisms are not adapted to fast-evolving product pipelines, which are essential for product development, but focus on investigator-led research consortia, which only suit basic research needs. The portfolio approach constantly reprioritizes the best and most viable candidates for clinical testing in and with developing countries and succeeds when it has the flexibility to contract with partners based on needs that emerge during the product development process.
- Perseverance is essential for succeeding in scientific research. We therefore urge the EU to make available long-term, flexible funding for global health product development, and to recognize the important role the public and private sectors can play in stimulating research for poverty-related and neglected diseases when market mechanisms fail.

SPECIFIC RECOMMENDATIONS CONCERNING EDCTP-II

PDPs strongly support the goal of EDCTP to build partnerships and to support and facilitate clinical trials in Africa. PDPs applaud the proposed increased budget for EDCTP-II as an important element in translational research funding. Given the growing significance of the EDCTP as an important mechanism for funding and building capacity for global health research, it is timely that its design and scope be revised so that a broader range of PDPs are able to effectively utilize this mechanism as a means to support the clinical evaluation of PDP-generated products.

We therefore request the European Union and Member States to consider the following changes in the EDCTP-II:

- Modification and more flexibility of the co-funding criteria.
- Extension to support clinical trials in other endemic countries outside of Africa.
- Expansion to other neglected and tropical diseases.
- Include support for Phase 1 through Phase 4 clinical activities.



KEY FEATURES OF PDPs

PDPs deliver life-saving, innovative new health tools.

During an historic period of R&D in global health, PDPs have become central to bringing forth solutions for treating, preventing, and diagnosing neglected diseases in poor countries. Nineteen products developed by PDPs have been launched in the last decade, including several major breakthroughs. Between 2000 and 2009, PDPs accounted for a growing share of all products gaining regulatory approvals to treat poverty-related and neglected diseases from 15 percent to 46 percent annually.⁴

PDPs offer value for money and reduce risks for public investment.

PDPs work on a portfolio basis, selecting winners and winnowing out non-performers; only the most promising candidates will be accelerated through the product development cycle. In total, these advantages help reduce the burden on public donors to fund the entire development process, and ensure that only the best products are developed. As they cover the full innovation cycle and manage partners of excellence, PDPs also tackle the issue of fragmentation.

Because the PDP R&D model is focused on developing products for the world's poorest people, donors minimize the risk of having to deliver inappropriate and expensive solutions that are not sustainable.

PDPs are part of the overall investment in health research that greatly boosts local and national economies. PDPs offer significant experience in working internationally with public and private partners in the EU, in developing countries, and in the rest of the world. As part of

this work, PDPs help build the capacity of people and institutions in developing countries, allowing countries to take more control of the product development in the future.

Global health R&D delivered by PDPs has much to contribute to economic growth and development and the fight against poverty. Health research and development can strengthen scientific and technological capacities in developing countries and, over the longer term, help diversify local economies, create jobs, stimulate economic growth, and reduce poverty.

How PDPs contribute to the realization of the EU Council conclusions on the EU role in Global Health (May 2010)

In the EU Council Conclusions on the EU role in Global Health, it was stated that the EU and its Member States should promote effective and fair financing of research that benefits the health of all. It was also highlighted that the EU should ensure that innovations and interventions produce products and services that are accessible and affordable.

PDPs already contribute in numerous ways to the EU's call on global health, as demonstrated in select examples below:

ad 18b ■ of Council conclusion: "to increase research capacities in public health and health systems in partner countries and strengthening cooperation between the EU and partner countries in this respect".

PDPs have created various research platforms, and they strengthen health capacities in developing countries in their daily work. They collaborate with developing country partners to advance

candidates in the R&D pipeline and invest significant resources in partner countries to support and expand the infrastructures in the communities in which they operate:

ad 18c ■ "explore models that dissociate the cost of Research and Development and the prices of medicines in relation to the Global Strategy and Plan of Action on Public Health, innovation and intellectual property, including the opportunities for EU technology transfer to developing countries"

ad 18d ■ "ensuring that EU public investments in health research secure access to the knowledge and tools generated as a global public good and help generate socially essential medical products at affordable prices, to be used through rational use"

ad 18.e ■ "strengthen and balance the complete health research process of innovation, implementation, access, monitoring and evaluation. International cooperation, common platforms of knowledge sharing and exchange of good practices are essential in this field."

PDPs were created specifically to address the research gap present in the development of products for diseases that lack viable commercial markets. PDPs use different models of IP management and licensing arrangements in their partnerships with academic institutions and private companies, aiming to de-link the costs of R&D from the cost of final products. Notwithstanding the different approaches to IP and licensing issues, the common goal of all PDPs is to accelerate the development of new medical products, and to ensure that these will be made available in developing countries rapidly after licensure, at affordable prices, and in sufficient quantities.

⁴ Tufts Center for the Study of Drug Development. Drug Approvals for Neglected Diseases Increase Along With More R&D Funding Impact Report. November/December 2009;11(6).