MedChild Foundation: A Nonprofit Model For Drug Development in Children.

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Abstract

The MedChild Foundation, is a non-for-profit public institution recently founded (http://www.medchild.org) whose main purpose is to promote an in-depth understanding of the conditions of children across the Mediterranean and to facilitate the exchange of knowledge, innovative policies and best practices, which will provide new ways for preventing, mitigating, or coping with children's exposure to social risks, and to promote children's rights and health within the framework of Euro-Mediterranean cooperation.

It is broadly acknowledged that the pediatric population requires special attention, in particular in the risk assessment of pharmaceuticals (drugs). Therefore, concern has grown over the last years regarding the widespread use of unlicensed and off-label medicines in children. The European Union estimates that approximately 50% of drugs used in children have never been actually studied in this population, while they were tested only in adults, and not necessarily for the same indications, or even the same disease. Lacks of information and appropriate pharmaceutical formulations to support a proper administration of medicines in children could expose such a vulnerable population to unwanted adverse effects or under-dosing without the expected efficacy.

Childhood usage is frequently a small proportion of a given drug's use, and a high cost is associated with obtaining pediatric labeling. Many promising drug candidates are discarded for lack of a viable market.

The MedChild Foundation by the establishment of a Pediatric Clinical Trial Office (PCTO) embraces the opportunity and responsibility of bringing these new medicines for children to light.

The MedChild Foundation PTCO includes among its main tasks the conduction of clinical trials and related translational research in pediatric therapeutics according to the Good Clinical Practice (GCP) and the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH).

The mission of the PCTO is to establish a platform for pediatric clinical trials in order to facilitates and promotes pediatric labeling of new drugs or drugs already on the market. In this process, the PCTO and MedChild Foundation strive to foster cooperative research efforts among academia, regulatory agencies, industry, and health professionals on international basis and within the framework of Euro-Mediterranean cooperation.

The MedChild Foundation catalyzes new drug development by creating opportunities:

- **For industry:** The pharmaceutical and biotechnology industries are the world's greatest sources of new drug leads. New drug development cannot succeed without the participation of industry, and the MedChild Foundation will apply a model that benefits industry and enhances their commitment to pediatric health.
- **For government and academia:** MedChild Foundation will provide the bridge between novel bench science and its conversion to applications for the developing world. We advocate to government and foundation funders in support of specific basic research that will later become new drug development projects. Advisors to MedChild Foundation include industry scientists who can advise academicians in translational science. MedChild Foundation can also serve as a bridge between academia and industry.

- **For developing world partners:** The developing world is not only a beneficiary, but also a tremendous resource that is often disregarded in new drug development. We are aimed at working with developing country partners in clinical trials, pharmaceutical manufacturing, and distribution of new medicines for neglected pediatric diseases. We are advocates for increased funding for academic laboratories in the developing world and we build capacity by training health care workers and scientists in clinical drug development through each of our projects. We will actively transfer knowledge and technology to improve local efforts to address disease threats. All of this work simultaneously fosters new avenues of economic development.

Overall, the MedChild Foundation PCTO will have capability to:

- Conduct Phase I - IV clinical pharmacology trials that conform to GCP/ICH regulatory requirements in a safe, effective and timely fashion;
- Produce data that has enabled expanded labeling of drugs for pediatric patients;
- Group academic pediatric clinical pharmacologists in the Mediterranean area who can provide expert consultation regarding protocol development and design;
- Bridge highly skilled pediatric clinical research professionals and sub specialists who work together to effectively take a study from concept/ protocol design through implementation, data analysis and report generation.

The MedChild Foundation PCTO's non-for-profit model will act in a spirit of partnership and collaboration that capitalizes on the specific talents and resources that each participant can bring to this essential scientific endeavor.