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HIGHLIGHTS

News and views

Peter Witters[1]

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Review on drugs in inborn errors of metabolism: the future is looking bright(er)

In developing and producing drugs for orphan diseases, there is an obvious contradiction between the rarity of the disease and possible financial gains associated with drug development and production if drug prices are kept low. Modalities aiming to tackle this contradiction include orphan drug legislation and the enthusiasm of drug developers.

In *Nature Reviews Drug Discovery*, a recent analysis shows that there are numerous orphan drugs in the pipeline in Europe (Morel et al. 2016). The European framework for orphan medicinal products offers a range of incentives to encourage development of these products. The analysis by Morel et al. revealed 502 orphan drugs still in development, with 316 having reached clinical development (35 % in phase I, 48 % in phase II, and 17 % in phase III). The largest subset (35.5 %) targets rare neoplastic disease, whereas 7.3 % (44) are relevant for inborn errors of metabolism. It is estimated that 90–110 of all these orphan drugs will reach market authorization. This analysis demonstrates the success of the European regulatory framework in promoting orphan drug development, which is driven (in 50 % of cases) by small- or medium-sized enterprises (Morel et al. 2016). It is widely acknowledged that crowdsourcing collaborations can increase dissemination of knowledge (e.g., Wikipedia) or development of open-source software (e.g., Linux). A similar methodology might also work in drug development. In *The Lancet*, an overview was published on open-source drug development that involves data sharing and collaboration and results sharing (Anderson 2016). However, while an army of coders can produce software for a fraction of development costs and time than can commercial rivals, drug discovery, development, and production are exponentially more difficult. To this end, the Open Source Pharma Foundation (OSPF) will develop open-source drugs and run the first open-source clinical trials. It plans to establish an alternative drug discovery route for neglected diseases where the research and development incentive is weak. While we are not aware of any drugs targeting inborn errors of metabolism, the idea of unleashing researchers with day jobs, who love problem solving or doing altruistic work in their spare time, seems promising. So far, the OSPF has not yet brought a drug to market, and its first clinical trials will probably focus on repurposed drugs (Anderson 2016).

Compliance with ethical standards

Conflict of interest None

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✉ Peter Witters
peter.witters@uzleuven.be

[1] Department of Paediatrics and Metabolic Center, University Hospitals Leuven, Leuven, Belgium