GlobalData

Drug repurposing (DRPx) is on the rise, and will lead to much-needed new treatments for rare diseases, and over time is expected to drive a steady rise in the number of orphan drugs derived from repurposing drugs, according to GlobalData , a leading data and analytics company.

The Orphan Drugs & Rare Diseases Global Congress 2018 Europe featured several discussions on drug repurposing for rare diseases. The discussions were led by Findacure CEO Rick Thompson and SOM Biotech Founder and President Raúl Insa and explored topics including the potential role, current extent and future development of drug repurposing.

DRPx is focused on de-risking therapeutic development, and is a promising field in drug discovery for rare diseases. Orphan drug indications are among the most profitable therapeutic areas within the DRPx market, as about 20% of orphan drugs are DRPx, and orphan CNS diseases provide a huge market potential for DPRx.

Hayley Chan, PhD, Healthcare Analyst at GlobalData , comments: A variety of unique benefits are offered by DRPx. Firstly, it reduces the development cost to one twentieth compared with

new chemical entities (NCE), which typically cost more than $1 billion, compared with less than $50m for DRPx. Secondly, it can reduce the time to market by half, at five years for DRPx compared with 14 years for NCE. Thirdly, the failure risk is dramatically reduced; for DRPx the risk rate is only 3:10, while for NCE it can be as high as 1:100000."

Orphan medicines, meanwhile, also offer several benefits in different regions, such as market exclusivity, protocol assistance, low regulatory fees, tax credits on clinical trials, subsidies for clinical trials, fast track or breakthrough designation, accelerated approval and accelerated assessment/priority review designation.

Chan continues: "Consequently, it appears an attractive investment opportunity for pharma companies to combine orphan drugs with DRPx."

Among the new therapies approved by the FDA, approximately a third are repurposed drugs, and it is estimated that 25% of annual pharmaceutical industry revenues comes from repurposed drugs, with the global DRPx market expected to continue to grow."

Chan concludes: "To date, there have been more than 11 DRPx companies acquired by larger

pharma companies, with a total investment of $2.3 billion in these deals. With the availability of new technologies and the advancement of big-data analytics, modeling software, and high-throughput screening techniques, it is anticipated DRPx will bring significant benefits for both pharma companies and patients with orphan diseases."