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REPURPOSING & COLLABORATIVE DRUG DEVELOPMENT FOR RARE DISEASES

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The label “rare/orphan disease” refers to diseases that affect a small number of people compared to common diseases. In this category, more than 7,000 diseases have been identified so far. 80% of them are of genetic origin and often affect children. A small fraction of these diseases have treatments, but often are very expensive. Due to rapid population migration in the last 3-5 decades, the genetic diseases are no longer confined to any one geographic location, just as infectious diseases such as malaria, TB, etc., are restricted to developing nations and are generally prevalent in poor communities. Rare diseases do not distinguish national boundaries; making them a concern to all nations—rich or poor, all races, religions and social affiliations. Therefore, orphan drug development need not and can’t be in any one country. It is a shared responsibility.

Pharmaceutical companies were reluctant to develop drugs for rare diseases under the assumption that they can’t make reasonable profit. In recent years this mindset has changed, thanks to the U.S. Orphan Drug Act (ODA), and recent developments in gene technologies. Under the incentives of the ODA, pharmaceutical companies are willing to develop drugs for rare diseases, and this signifies an increasingly important component of the pharmaceutical market; but, they are slow in taking advantage of academic knowledge, new technologies and the power from the advocacy of patient populations. Only 5% of rare diseases have treatments to date!

To shorten the time of drug development from the normal 12-15 years to 3-4 years, and cutting the research costs to one third, we believe drug

repositioning/repurposing is one of the preferred ways. However, repurposing should not be viewed as a substitute for traditional drug discovery. It is partly motivated by a need to reduce but not eliminate the risk. The current rate of drug repurposing activity raises the expectation that a substantial percentage of rare diseases, if not all 7,000 rare diseases, might be treatable with drugs in the current pharmacopeia. In this endeavor, NIH, FDA, pharmaceutical and biotechnology companies, rare disease foundations, academic drug discovery centers, and the public have been collaboratively working for the last 4-5 years. The strategy of targeting rare diseases has been one of the most successful in the drug industry over the past decade. The repurposing of drugs and drug-like molecules should be anticipated to have a promising future.