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ICER Report Further Justifies Gene Therapy for Sickle Cell Disease and Other Inherited Disorders

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INTRODUCTION

Recent report by the Institute for Clinical and Economic Review (ICER) [1], a clinical cost effectiveness watchdog based in the United States of America, provides further significant justification for health policy support in aid of Gene Therapy for Sickle Cell Disease (SCD) and similar inherited disease conditions. Sickle Cell Disease is an inherited (genetic) blood condition characterized by several debilitating symptoms, principally sudden painful episodes of vaso-occlusive crises that can lead to irreversible damage to several organs [2]. The ICER report relates to the initial enthusiasm and hope for improved quality of life for SCD patients generated by the approval of the new drugs Oxbryta (GBT) and Adakveo (Norvatis) but the enthusiasm was dampened by the comparative advantages of Gene Therapy in terms of outcome for patients, especially those in rural and remote places already hampered by financial and geographic access to effective health care [3,4].

Upon approval of the new drugs, the estimated cost of treatment with Oxbryta per patient per annum was $127,000 and for Adakveo was $132,000 per year. ICER in its report suggested that a more appropriate price for the drug should be much lower [5,6], additionally, that a fair price should be $8,300 to $36,500 for Oxbryta and $16,900 to $52,000 for Adakveo per year in the United States of America, where it is estimated that over 100,000 patients are affected by SCD [2]. In many other parts of the world, particularly resource constrained areas were the disease burden is much higher [4,7], the suggested appropriate pricing by the ICER report will amount to catastrophic expenditure for healthcare for many households.

Importantly, these new drugs offer an improved quality of life for SCD patients, particularly where fears that comorbidity with Corona Virus Type 2 infections may worsen instances of tissue oxygen perfusion that the drug Oxbryta may likely improve. Still, what they offer is an improvement in a chronic situation for the SCD patients and their affected families. On the other hand, Gene Therapy offers not just a marginal improvement in quality of life but the prospects of a cure, with promising advancements in recent years, such as gene editing and disabling of the BCL11A gene [3,8,9], as well as effective and minimally invasive options of vector delivery also being researched [10]. Provided with an option for a cure rather than an interminable catastrophic expenditure, the ICER report is further correlating justification for wider policy and funding support for Gene Therapy options for SCD patients and other inherited disorders that may gain from SCD gene therapy

research, these are important to augment the primary health strategies of pre-marital screening and counseling, particularly in rural and resource constrained areas where financial and geographic access are key concerns that will be worsened if these drugs are offered at such cost. Importantly advances in Gene therapy for SCD will also advance similar treatments for related inherited diseases.

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