CSIR-CCMB

Hydroxyurea gets DCGI approval for Sickle Cell Anemia treatment

Hyderabad, 23rd Dec, 2021: Sickle Cell Anaemia (SCA) is a common genetic disorder among Indians affecting the red blood cells. It is transmitted by parents who carry a defective beta globin gene without suffering themselves. Close to 0.4% of the population suffers from the disease while 10% people are carriers who lead to birth of new SCA patients. The disease is well-known in tribal populations as well as prevalent in general populations in states like Maharashtra, Madhya Pradesh, Chhattisgarh and Orissa. The disease starts early in life, and the affected children have persistent pain, low amount of haemoglobin (anaemia), low energy, reduced growth plus other abnormalities and multiple episodes of frequent severe pain better known as Vaso-occlusive crisis.

Like most of the genetic disorders, SCA has no cure but has symptomatic treatments for pain, anaemia and Vaso-occlusive crisis. One of the rather inexpensive drugs, hydroxyurea, largely used as an anti-cancer agent is also used in SCA treatment without any formal approval. The commercially available hydroxyurea formulations are made with its anti-cancer role in mind, and hence, are of big quantity size (minimum 500 mg). SCA children are typically of low weight, and consequently, their dosage size needs to be much smaller. Given the fixed and larger size of currently commercially available hydroxyurea capsules, it is difficult to give the correct doses to SCA patients. Although, there is robust response to hydroxyurea therapy, the complexity and cumbersome nature of disbursing the right dose leads to low compliance and sometimes unpredictable response.

Under CSIR-Sickle Cell Anaemia (CSIR-SCA) Mission with 6 CSIR labs and 3 Government hospitals in Chhattisgarh, Madhya Pradesh and Maharashtra, scientists and clinicians are trying to address various lacunae in SCA diagnosis and disease management. The focus is on identifying the patients through population-based screening in states with high disease prevalence and helping the family with proper treatment and preventing the disease in the next generation. One of the major objectives of the Mission has been obtaining approval for use of hydroxyurea for treatment of SCA. CSIR-SCA Mission, coordinated by the CSIR-Centre for Cellular and Molecular Biology (CSIR-CCMB) with the help of Cipla, one of the manufacturers of Hydroxyurea and with active support from CSIR-IIIM, approached the Drugs Controller General of India for approval of hydroxyurea for SCA treatment. On 9th Dec, 2021, a committee of experts constituted by Central Drug Standard Control Organisation (CDSCO) critically evaluated the proposal and approved marketing of hydroxyurea for treatment for SCA, subject to Post-marketing Surveillance.

The approval currently legalises the drug to be used at standard doses for treatment of SCA. It also sets up stage for designing various formulations of smaller dose sizes that promise higher compliance rates in SCA children and may even lead to syrup-based formulations. “This is a landmark achievement for Sickle Cell Anaemia community. This adds to the advantages of identifying the patients through a targeted screening program. While one of the major focuses of the screening program is to avoid birth of affected children through genetic and social counselling, this approval provides comprehensive treatment to the identified patients. The message now needs to reach clinicians across the country so that they can use Hydroxyurea regularly for their patients”, says Dr Giriraj R Chandak, Chief Scientist at CSIR-CCMB and Mission Director, leading the CSIR-SCA Mission.