Friday, April 7, 2023

WHO EML Expert Committee
emlsecretariat@who.int

Dear esteemed WHO EML Expert Committee Members,

I am writing to you on behalf of the Thalassemia Community in Canada appealing for your kind consideration in favor of equitable and sustainable access to Deferiprone (an oral iron chelation agent) for thalassemia and all other chronically transfused patients. We wish to express our support for the "Proposal for the Inclusion of Deferiprone in the WHO Model List of Essential Medicines for the Treatment of Transfusional Iron Overload in Adult and Pediatric Patients with Thalassemia Syndromes, Sickle Cell Disease or Other Anemias" submitted by Chiesi Farmaceutici S.p.A dated December 2022.

The Thalassemia Foundation of Canada (TFC) is a patient driven support organization serving the thalassemia community across Canada. The TFC started as a patient and parent peer support group back in the eighties and has grown to become a national patient organization leading activities and objectives that benefit all thalassemia patients. TFC has been a registered Canadian charity since 1988. The mission of TFC is to support and fund thalassemia scientific research, treatment, patient services, public awareness and education.

In addition, TFC has been a long standing and prominent member of Thalassemia International Federation (TIF), collaborating on common goals to bring health equity and equality for sustainable access to comprehensive care for Thalassemia worldwide.

Hemoglobin disorders are hereditary and chronic disorders with varying degrees of severity that need multifaceted management. In the most severe form, death in infancy is inevitable without treatment. Thus, early diagnosis and comprehensive care programs that include access to life saving therapies are essential to sustain survival and allow patients to live a close to normal life.

Thalassemia disorders are genetic blood disorders where the patient's body does not produce sufficient red blood cells. Patients living with the severe forms of thalassemia have life dependency on regular red blood cell transfusions, which causes iron overload. If untreated,

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results in progressive organ dysfunction and premature death. Because of iron overload, many thalassemia patients suffer from comorbidities such as heart and liver disease, pulmonary hypertension, diabetes, and other endocrinopathies that are known to reduce survival.

Fortunately, and thanks to medical advances, the use of iron chelation agents over the past several decades has been instrumental in preventing serious health complications and comorbidities that would add to an already disease burdened patient and family. However, not all iron chelation agents are equally affective for all thalassemia and other chronically transfused patients. Often chelation agents cause harmful undesired side effects such that alternative chelation agents must be prescribed.

In addition, it is naturally understandable and evidently proven that better adherence to treatment is achievable through oral iron chelation than a cumbersome self-injections regimen alternative. It has been demonstrated and well known that patient outcomes could be improved through equitable and sustainable access to a variety of available iron chelation options for thalassemia and other chronically transfused patients.

Deferiprone, while it has been licensed and accessible to thalassemia patients living in Europe and North America, it remains a challenge to access this life saving medicine in many parts of the world. This life-saving medication which has been proven to be a very affective iron chelation agent is prescribed to patients who could not tolerate or benefit from using other available iron chelation options which are already on the EMLc. Expanding an equitable and sustainable access to various chelation agents is what thalassemia and other chronically transfused patients deserve rather than limiting treatment options because of access denial that would burden patients and their families with the high costs of life saving therapies.

We applaud WHO's decisions in 2021 to include insulin and oral diabetes medications on the Model Lists of Essential Medicines and Essential Lists for Children. However, we feel that it is unjust, rather discriminatory not to include all available oral iron chelation medicines on the same list. Like insulin and oral diabetes medications, Deferiprone and other chelation therapies are essential to sustain life and prevent further complications and comorbidities for thalassemia and chronically transfused patients.

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We ask the honorable committee members to grant recommendations to include Deferiprone in the WHO Model List of Essential Medicines for the treatment of iron overload for patients living with Thalassemia and other chronically transfused patients. We believe this will be a major step in favor of an equitable and sustainable access to Deferiprone for thalassemia and other chronically transfused patients that will expand the choices for life saving oral chelation therapy options, hence will effectively improve patient outcomes globally.

We look forward to receiving the Committee's recommendations and we greatly value the opportunity to express and share the views and position of the Thalassemia Community.

Sincerely,

Riyad Elbard,
President

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