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Editorial

Thalidomide in Thalassemia: A Fortune in Making

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Thalidomide is the unfortunate drug which got entangled in the greatest iatrogenic catastrophe in human history[1]. In 1950s, deemed safe and "miraculous", the drug was prescribed to pregnant women with nausea and insomnia. By that time nobody knew about its teratogenic potential. This culminated in around 10000 births with severe morbidities. In the aftermath, thalidomide was banned immediately all over the world[1].

The drug however, never seized to loose attention. The first rational indication was identified for Leprosy by Dr Jacob Sheskin in 1964[2]. In 1990s, Dr Judah Folkman demonstrated utility of thalidomide in treating Multiple Myeloma[3]. These indications portray the anti-inflammatory, immunomodulatory and anti-cancer properties of the drug, which have been pursued in other similar diseases as well. At current, literature reports efficacy of thalidomide in several dermatological, rheumatological, gastrointestinal, cardiac and malignant diseases[4]. Of particular significance is the role in thalassemia, a well-known haematological disease.

Since 2004, about a dozen studies have reported role of thalidomide in thalassemia patients. These include studies from Mexico, Italy, China, Iraq, India and Pakistan; comprising data of 440thalassemia patients. Results from a formal clinical trial are, however, scarce, with only one such study from China depicting phase-1 results in 22thalassemia patients [5]. All these studies advocate a significant improvement in the baseline haemoglobin (Hb) levels of thalassemia patients.

Thalidomide is cheap drug, costing around 5-10$ a month in an average patient at an average dosage. On the other hand, in a comparable patient, regular transfusion covered by adequate chelation, costs around 60 to 80$. This is in addition to the mental and social stress brought about by transfusions [6].

Luspatercept, marketed by the name of Reblozyl® is a new drug with comparable effects in TDT patients[7]. Safety profile and efficacy of the two drugs are comparable for thalassemia patients[8,9]. Luspatercept, being FDA approved, is the preferred therapeutic agent for TDT patients. The key limitation, however, is its price ($3,441 per 25 mg vial) which takes it out of equation in local scenario [10].

In Pakistan, being a country with inadequate health care resources and apropensity for consanguinity, approximately 9000 children are born with thalassemia each year[11]. According to local statistics, around 40% of transfusions in Pakistan are necessitated by thalassemia patients[12]. This poses a great deal of burden on national health care system. The mean age of Pakistani TDT patients range to around 10 years [13,14]. This reflects the incapability of our health care system to take good care of these patients. With these constraints in view, thalidomide is a light of certain hope. Thalassemia is no more a major concern in developed countries for they have already excelled in thalassemia prevention programs. Role of thalidomide in thalassemia needs to be elucidated by the developing nations, on their own. Health authorities, medical research institutes and organizations dealing with thalassemia management need to pay serious attention to this novel therapeutic option. With so much at stake, Pakistani researchers ought to prioritize this quest and lead from the front.

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