Study of Efficacy, Toxicity, and Cost of Deferasirox in Adult Thalassemic Patients with Iron Overload under EXPAP Program at Siriraj Hospital

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Background: Iron overload is a major problem in thalassemic patients. Thus, iron chelator is essentially needed. More potent, oral iron chelator and deferasirox (DFX) has shown more efficacy and safety than conventional deferoxamine (DFO) or oral deferipone (DFP). Exjade Patient Assistant Program (EXPAP) has recently initiated to provide coverage of costly DFX to patients who previously cannot access to this drug financially.

Objective: We aimed to study the efficacy, toxicity and cost of deferasirox in adult thalassemic patients with iron overload under EXPAP Program at Siriraj Hospital.

Methods: Adult thalassemic patients under EXPAP program in Siriraj Hospital during 2014-2017 were retrospectively reviewed for demographic data, iron overload status, reasons for chelator change, efficacy, toxicity, and economical value, which compared before and after changes.

Results: Of 25 registered patients, 2 were excluded due to death prior to drug administration (1) and non-thalassemic (1). Remaining 23 patients were HbE/beta-thalassemia (16), HbH (2), HbH/CS (1), AEBart (1), AEBartCS (1), homozygous beta-thalassemia (2) with transfusion-dependence (TDT) (20), and post-splenectomy (17). Prior chelator use was DFO (6), DFP (11), combined DFO, and DFP (4) or none (2). Previous organ impairment was DM (1), AF (1), cirrhosis (4), congestive heart failure (1), hypogonadism (2), hypoparathyroidism, (1) and subclincal hypothyroidism (3). Reasons for change were inability to reduce ferritin to <1000 ng/ml (23), elevated liver enzymes (3), refractory neutropenia (1), arthralgia (2), severe nausea and vomiting (4), and injection device unavailability (1). Changed chelators were DFX (8), DFX/DFO (10), DFX/DFP (3), and DFX/DFO/DFP (2). Mean DFX dosage (range) was 1221 (498 – 2057) mg/day. Mean ferritin levels (range) at baseline, 6, 12, and 24 months were 3488.34 (1090 – 6808), 3278.73 (334 – 8818), 3208.53 (249.20 – 7413), and 3607.03 (419.20 – 8271) ng/ml, respectively. Despite overall statistically insignificance, 3 of 8 DFX patients and 3 of 15 combined chelators promisingly showed reduced ferritin level to <1000 ng/ml. Toxicity were transaminitis (17), diarrhea (7), nausea/vomiting (5), acute kidney injury (5), abdominal pain (4), and rash (4). Chelator cost prior to and after DFX change was statistically insignificant.

Conclusion: DFX use in thalassemic patients under EXPAP program demonstrates non-inferior efficacy and cost with tolerable toxicity and oral-route convenience.

Keywords: Iron overload, Thalassemia, Deferasirox, Deferoxamine, Deferiprone, EXPAP Program