

## Granulocyte colony-stimulating factor therapy for facioscapulohumeral dystrophy: a case report

Sienkiewicz D.<sup>1 A,B,D,E,F</sup>, Kułak W.<sup>1 A,D,F \*</sup>, Okurowska-Zawada B.<sup>1 E,F</sup>, Paszko-Patej G.<sup>1 E,F</sup>, Wojtkowski J.<sup>1 B,F</sup>, Dmitruk E.<sup>1 B,F</sup>, Okulczyk K.<sup>1 B,F</sup>, Sochoń K.<sup>1 B,F</sup>, Kalinowska A.<sup>1 B,F</sup>, Żak J.<sup>2 B,F</sup>, Pogorzelski R.<sup>3 B,F</sup>

1. Department of Pediatric Rehabilitation, Medical University of Białystok, Poland
2. Department of Pediatric Laboratory Diagnostics, Medical University of Białystok, Poland
3. Department of Neurology, Medical University of Białystok, Poland

---

A - Conception and study design, B - Data collection, C - Data analysis, D - Writing the paper, E - Review article, F - Approval of the final version of the article

---

### ABSTRACT

---

We examined the safety and effectiveness of a low dose of analog granulocyte-colony stimulating factor in a 15-year-old boy with facioscapulohumeral dystrophy. The onset of disease was noted at 12 years of age. The physical examination noted general muscle atrophy more pronounced at left side of the body. He was able to walk 300 meters within 6 minute walk test. Granulocyte colony-stimulating factor 5 µg/kg was given subcutaneously daily for 5 days/month for 1, 2, 3, 6 and 12 months. Clinical examination, laboratory tests including blood, biochemical tests, and CD34+ cells were performed. A significant increase of muscle strength in the lower and upper limbs between baseline, and after 3 months of treatment, after 6, and after 12 months was found. He was able to walk 480 meters within 6 minutes

after 12 months. Electromyography demonstrated increase of amplitude in the examined in upper and lower limbs after six months compared to baseline. Leukocyte levels remained below 25000/µL. CD34+ increased significantly at day 5 of granulocyte colony-stimulating factor administration. It was safe and well tolerated by the patient. A significant increase in muscle strength in this patient with facioscapulohumeral dystrophy after 3 months of treatment, after 6, and after 12 months since the first treatment course was completed may indicate beneficial effects of granulocyte colony-stimulating factor in this disorder.

**Key words:** Facioscapulohumeral dystrophy, granulocyte colony-stimulating factor, muscle strength

---

DOI: 10.5604/01.3001.0009.5165