

**IMPACT – Improvement of Motoric Abilities in  
Patients with Cystinosis Project report summery**

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In patients with cystinosis the condition of the musculature is important for the course of the disease. Regular exercise builds muscle and can positively influence life expectancy and quality of life. IMPACT aims to show whether regular exercise with vibration plates increases muscle strength in cystinosis patients, to reduce serious medical interventions in the long term and to improve quality of life. To do so, the study uses a training concept, based on the established approach of pediatric rehabilitation “Auf die Beine”, developed for children and adolescents with limited mobility, where participants exercise while standing on a Galileo vibration platform. For comparison a second group exercises only their arms using a vibration dumbbell. Both exercise according to a fixed training schedule with 10 short training sessions per week. Each training session lasts between 5 and 8 minutes. The training program is tailored to the disease, and the dumbbell exercises are simplified for the younger children. Training at home and the short duration aims to facilitate integration into everyday life.

To assess the concept, patients are asked to document their training progress as well as any difficulties or side effects. During the exercising period patients are contacted via video to check on training progress and to address potential problems. In addition, the training is accompanied by a baseline and three follow-up clinical assessment, one after the three-month training phase, one after a three-month follow-up phase, and one after one year. Participants are provided with step counters to measure daily activity and a quality-of-life questionnaire before each assessment. During the assessments muscle strength and cardio-respiratory function are measured. Additionally, at baseline and after one year, a physiotherapeutic and orthopedic examination and routine clinical and laboratory evaluations are performed.

Twenty-four adult patients from Germany and Italy, participated in the Study between September 2020 and September 2021. In addition, fifteen juvenile patients (between 5 and 12 years) from Germany, Austria, and Slovakia started their study in September 2021 and will finish in September 2022. Based on sex, age, and kidney

transplant status, patients were randomized into the group using the vibration platforms are the one using the vibration dumbbell.

The first analysis of the training diaries shows, that 20 out of the 23 adult participants, who completed the training period, completed at least 80% of the required 10 training sessions per week, 15 of them even more than 90%. The adult participants reported no negative effects during the training sessions (injuries or pain due to the training). Out of the 14 children, who completed the training period, 11 met at least 80% of the required training sessions, 9 met more than 90%. Even though they performed simplified exercises and used dumbbells, designed for children, the training with the dumbbell was difficult for 4/15 children. Parents also had to help more with the exercises with the dumbbells than with the platforms. Nonetheless, parents of both groups gave positive feedback and repeatedly reported visible improvements in their children. The results of the comprehensive statistical analysis for the adult group are expected in June 2022, results for the juvenile subgroup at the end of the year.