

Title: THE SWEDISH CF REGISTRY FACILITATES THE EVALUATION OF ORKAMBI® TREATMENT

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Objectives: Starting July 1, 2018, the Dental and Pharmaceutical Benefits Agency decided that Orkambi® was to be subsidized by the Swedish state. The decision was accompanied by the following restrictions: 1. Treatment initiated at a CF Centre with close follow-up of effect and adverse events 2. Set treatment goals evaluated after one year 3. Follow-up via the Swedish CF Registry. The aim was to investigate whether the registry could facilitate the evaluation of Orkambi® treatment.

Methods: A national follow-up program for the first year of Orkambi® treatment was established by The Swedish CF Working Group. Extra fields were added to the registry to fit the program and staff was hired to ensure that data was entered correctly. Mandatory check-ups were at treatment start and after 1, 3, 6, 9 and 12 months.

Results: The registry is used by all 4 centres and all patients that initiated Orkambi® are included in the follow-up program. Up to July 31, 2019, 190 patients entered the program. 25 (13%) patients stopped treatment due to adverse events. Mean age (SD) at start was 22.9 (12.7) yrs with FEV1pp of 77 (22)% and LCI (<18 yrs) 8.89 (1.99). BMI in adults was 21.9 (2.51) and BMI z-score in children -0.35 (0.82). 87 patients (35 children) had been on treatment for >6 months July 31, 2019. The sweat test decreased in average 18 mmol/l (range -48 to +14 mmol/l) after 3 months. After 6 months, FEV1 showed a trend of improvement in the adult population while it was stable in the pediatric population (6-18 yrs) where a decrease of LCI (0.7 units) was seen. Both BMI and BMI z-score showed a slight increase. Comparing the 180 days before starting Orkambi® to the 180 days after treatment start, the mean number of days on iv antibiotics per patient decreased from 7.2 to 3.1 days.

Conclusion: The follow-up program for Orkambi® shows that the Swedish CF-registry is a useful tool for evaluation of new therapies. Longer follow-up time will be presented in June 2020.