# EAHAD 2019 - Abstract Submission

*Regular abstract*

EAHAD19-ABS-1326

## PERSONALISED PROPHYLAXIS WITH SIMOCTOCOG -ALFA (HUMAN-CL RHFVIII) : A REAL-LIFE EXPERIENCE ON CHILDREN AND MODERATE HAEMOPHILIA A PATIENTS.

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**Introduction:** Human-cl rhFVIII launched in Italy in June 2015, is currently adopted in all segments and different ages: prophylaxis, on demand, surgery and ITI treatments. Results from NuPrewiq study showed the efficacy of personalized prophylaxis in adults with severe haemophilia A. We here report results of personalized prophylactic treatment with Nuwiq® (simoctocog-alfa) adopted also for children and patients with moderate haemophilia A

**Methods:** We have analyzed available data related to adolescent and moderate haemophilia A patients receiving a prophylactic regimen with human-cl rhFVIII, defined after pharmacokinetic (PK) studies (either individual or population PK). All the subjects treated by our Reference Regional Center for Haemophilia with available 12 months follow-up after starting a personalized prophylaxis regimen. Secondary aim of the study was to evaluate any change in health-related quality of life as determined by EQ-5D (adults), EQ-5DY-3L (for children) and EQ-VAS (routinely administered in our Center at each regular follow-up visit, since 2016).

**Results:** : Five patients met the above defined criteria for the current analysis: 2 children (7 and 13 yo) with severe haemophilia A and 3 adults with moderate haemophilia A and target joints. Prophylaxis was personalized after individual PK in 2 cases and population PK (WAPPS-haemo) in 2 children and one adult. At 12 –months follow-up, the median dosing interval during personalized prophylaxis was 2.5 days, with all adults on 2 weekly dosing. Mean annual bleeding rates during personalized prophylaxis were 2.5, of note bleeding occurred only in adults with arthropathy under secondary prophyaxis while childrenunder primary prophylaxis did not experience any breakthrough bleeding. Health related quality of life scores showed an overall improvement at 12-months, with particular reference to the following parameters: mobility and depression dimension for adults and usual activities dimension in children.

**Discussion/Conclusion:** The current 12-months follow-up analysis confirms the efficacy of a personalized treatment approach with human-cl rhFVIII also in children and subjects with moderate haemophilia A.

**Disclosure of Interest**: M. Napolitano Consultant for: Novonordisk, Bayer, , Speaker Bureau of: Octapahrma, Shire, BioFVIIx, S. Raso: None Declared, M. F. Mansueto: None Declared, A. Passannanti: None Declared, L. LoCoco: None Declared, M. Sardo: None Declared, S. Siragusa: None Declared