STUDY OBJECTIVES
Document baseline Quality of Life (QOL) for haemophilia patients with inhibitors and examine changes during a trial (MAA-201) of subcutaneous marzeptacog alfa (activated), a novel FVIIa prophylactic agent

CONCLUSIONS
→ The MAA-201 trial has demonstrated a significant reduction in proportion of days with bleeding using subcutaneous prophylaxis
→ Please attend the oral presentation OR11 on Friday 08 February 2019 08:30-10:00
→ Subjects entering MAA-201 had worse QOL scores at baseline than reported scores for patients without inhibitors almost uniformly across domains
→ After as few as 50 days of daily subcutaneous treatment with MarzAA, HAL and Haem-A-QOL scores were numerically improved compared with baseline

INTRODUCTION
→ Haemophilia A or B morbidity increases throughout life
→ Patients who develop neutralizing antibodies (inhibitors) (HPWI) to replacement clotting factor typically receive bypassing agents for episodic treatment of bleeds
→ The short half-life of available agents for HPWI means prophylaxis is infrequently utilized, resulting in subjectively worse QOL, worse musculoskeletal outcomes and significantly higher mortality when compared with patients without inhibitors
→ HPWI deserve improved prophylaxis
→ QOL in hemophilia may be evaluated by Haem-A-QOL and impaired physical activity with Haemophilia Activity List (HAL)
→ There is little data on QOL of HPWI compared with the broader population of hemophilia patients

METHODS
→ We studied Marzeptacog alfa (activated) (MarzAA) an engineered rFVIIa with 4 amino acid substitutions and 9-fold greater potency than wild-type FVIIa given daily subcutaneously for prophylaxis in inhibitor subjects
→ We evaluated the baseline scores of subjects in the MAA-201 trial using Haem-A-QOL and HAL and compared the results with those of subjects with severe haemophilia but without inhibitors recruited into a long-term prophylaxis trial (A-LONG) and to published data
→ QOL scores were re-evaluated after 50 days of subcutaneous MarzAA and compared with those at baseline for 4 subjects who have completed the trial

BASELINE RESULTS
→ Almost uniformly across domains, regardless of which QOL tool was used, subjects in MAA-201 had worse baseline scores than patients without inhibitors
→ Mean baseline Haem-A-QOL summed score for A-LONG was 29.3 ±15.7 contrasting sharply with a much worse mean baseline summed score of 44.8 ±20.0 in the MAA-201 trial
→ Using the more function-oriented HAL, MAA-201 median baseline scores were inferior across most domains compared with a reference population score

REFERENCES

DISCLOSURES
H. Levy & F. Del Greco: Employees of: CATALYST BIOSCIENCES, F.V. McL. Booth paid consultant to CATALYST BIOSCIENCES, J. Mahlangu Consultant and grant support from: CATALYST BIOSCIENCES

PATIENTS WITH INHIBITORS AT BASELINE IN MAA-201 HAVE WORSE MEAN HAEM-A-QOL SCORES COMPARED WITH PATIENTS WITHOUT INHIBITORS IN A-LONG

RESULTS FOR FOUR SUBJECTS AFTER TREATMENT IN MAA-201

AFTER 50 DAYS OF TREATMENT IN MAA-201 THE MEAN HAEM-A-QOL SCORES TRENDED TOWARDS IMPROVEMENT COMPARED WITH BASELINE SCORES

AFTER 50 DAYS OF TREATMENT IN MAA-201 THE MEAN HAEMOPHILIA ACTIVITIES LIST DOMAIN SCORES TRENDED TOWARDS IMPROVEMENT COMPARED WITH BASELINE SCORES