

PO106

## EMICIZUMAB PROPHYLAXIS IN SEVERE HAEMOPHILIA A WITHOUT INHIBITORS: OUTCOME AND INDICATIONS. FROM THE UK HAEMOPHILIA CENTRE DOCTORS' ORGANISATION

C. Hay<sup>1,2,\*</sup>, C. Wall<sup>3</sup>, H. Xiang<sup>4</sup>, B. Palmer<sup>4</sup>, P. Chowdary<sup>5</sup>, P. Collins<sup>6</sup>, S. Fletcher<sup>7</sup>, M. Mathias<sup>8</sup>, C. Percy<sup>9</sup>, P. Sartain<sup>10</sup>, S. Shapiro<sup>7</sup>, D. Stephensen<sup>11</sup>, K. Talks<sup>12</sup> on behalf of UK Haemophilia Centre Doctors Organisation

<sup>1</sup>Dept of Haematology, University of Manchester, <sup>2</sup>UK National Haemophilia Database, <sup>3</sup>Haematology, Manchester Royal Infirmary, <sup>4</sup>UKHCDO, UK National Haemophilia Database, Manchester, <sup>5</sup>Haematology, Royal Free Hospital and Medical School, London, <sup>6</sup>Haematology, University of Wales Hospital, Cardiff, <sup>7</sup>Haemophilia Centre, Churchill Hospital Oxford, Oxford, <sup>8</sup>Haemophilia Centre, Great Ormond Street Hospital for Sick Children, London, <sup>9</sup>Haematology, University Hospital Birmingham, Birmingham, <sup>10</sup>Patient Rep, UK Haemophilia Society, London, <sup>11</sup>Physiotherapy, Kent and Canterbury Hospitals, Canterbury, <sup>12</sup>Haematology, United Newcastle Hospitals, Newcastle upon Tyne, United Kingdom

**Introduction:** The indications for emicizumab in people with severe haemophilia A (psHA) without inhibitors are still debated and practice is diverse. We present UK outcomes 32 months after introduction for this group.

**Methods:** We conducted an observational study of 809 psHA using emicizumab 01/08/2019-31/03/2022. Within-person comparison of annualised bleed (ABR), joint bleed (AJBR) and spontaneous bleed (ASBR) rates with previous rFVIII prophylaxis was conducted in people with  $\geq 6$  mths. pre-and post-switch Haemtrack home-therapy diary data (Wilcoxon signed rank test). Change in % with zero treated bleeds was analysed using the McNemar test. Change in paired haemophilia joint health scores (HJHS) were analysed in a subgroup.

**Results:** Emicizumab was prescribed to 809 non-inhibitor psHA, 43 of all UK psHA. All subjects used prophylaxis before switching. A within-patient comparison with previous rFVIII treatment was conducted in 519 psHA. The zero treated bleed rate increased after switching from 35% to 71% ( $p < 0.001$ ). The median (IQR) ABR and AJBR reduced from 2.2 (0.4, 6.1) and 1.1 (0.3, 3.8) to 0.0 (0.0, 0.8) and 0.0 (0.0, 0.4) respectively ( $p = 0.001$ ). ASBR decreased from 0.7 (0.0, 2.8) to 0.0 (0.0, 0.0). A within-person sub-analysis of 197 psHA reporting bleeding after switching to emicizumab showed a median (IQR) change in ABR of -2.5 (-7.6, -0.2). Of these, 79% (147/187) bled less after switching ( $p < 0.001$ ). A total of 57 active target joints (using ISTH criteria) were identified in 38 psHA (7%). Of these, 49 (86%) resolved after switching. A clinically significant improvement in total HJHS (reduction  $\geq 4.0$ ) was seen in 24%; 61% had no change ( $< 4.0$ ) and 15% deteriorated (increase  $\geq 4.0$ ). The group median HJHS reduced from 9.6 (1, 29) to 8.5 (0, 28)  $p = 0.03$ .

**Discussion/Conclusion:** Switching to emicizumab was associated with a significant improvement in bleed control in all age groups of psHA without inhibitors. Most psHA had no post-switch spontaneous bleeds. Those who continued to bleed had significant reduction in ABR, AJBR and ASBR. Target joints tended to resolve and HJHS improved or was stable after switching in 85% of psHA..

**Acknowledgement:** This study was supported By Roche.

**Disclosure of Interest:** C. Hay Grant/Research support from: Research funding and study sponsorship from Roche, Speaker Bureau of: Roche, Sobi CSL, C. Wall Grant/Research support from: Study funded by Roche, H. Xiang: None declared, B. Palmer: None declared, P. Chowdary Grant/Research support from: sobi, CSL, Biomarin, Bayer, Speaker Bureau of: Sobi, CSL, P. Collins: None declared, S. Fletcher: None declared, M. Mathias: None declared, C. Percy: None declared, P. Sartain: None declared, S. Shapiro: None declared, D. Stephensen: None declared, K. Talks: None declared