

CHARCOT 2018

## **Ocrelizumab Compassionate Use Program for Patients with Primary Progressive Multiple Sclerosis in Germany**

Short Title: **Ocrelizumab CUP for Patients with PPMS in Germany**

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### **Introduction**

Approximately 15% of patients with multiple sclerosis (MS) suffer from primary progressive multiple sclerosis (PPMS). The anti-CD20 antibody ocrelizumab reduced the risk of confirmed disability progression with clinically meaningful efficacy in the double-blind, placebo-controlled phase III trial ORATORIO. In January 2018, the European Union (EU) approved ocrelizumab as the first therapy for patients with PPMS.

### **Objective**

Description of a Compassionate Use Program (CUP), including patient characteristics, which provided pre-approval access to ocrelizumab for patients with PPMS in Germany.

### **Methods**

The CUP started in February 2017 and ended in January 2018 with the regulatory approval of ocrelizumab for patients with PPMS and RMS.

Inclusion criteria: Adult patients with PPMS (McDonald 2010); positive benefit/risk ratio for ocrelizumab based on the estimation of the treating physician; contraception during treatment and  $\geq 6$  months after last dose; confirmed consent.

Main exclusion criteria: Current/recent treatment with other immune therapies; unresolved/chronic or active infections; severely immune-compromised status; suspected or confirmed progressive multifocal leukoencephalopathy; current/planned vaccinations within 6 weeks prior to program start; pregnancy/breast feeding; antineoplastic therapies; severe organ diseases.

Participating patients received premedication (methylprednisolone, antihistamine) followed by 600 mg intravenous ocrelizumab in 6-month cycles. Adverse events (AEs), serious AEs, AEs of special interest and pregnancies were reported immediately.

### **Results**

From 580 patient requests (104 centers), 525 patients fulfilled eligibility criteria. 35 patients did not participate due to withdrawal by the treating physician, 1 patient due to death.

In total, 489 patients received the first ocrelizumab cycle and 51 received a second cycle. 49% of the patients were female. Median age was 52 years (range: 24–73). AEs, previous diseases and treatments will be presented.

### **Conclusion**

This CUP provided access to ocrelizumab for 489 patients with PPMS in Germany up to 11 months prior to ocrelizumab approval in the EU. Reported AEs were consistent with published data.