STUDY RATIONALE

• All eligible patients will be invited to participate in the study at the time of their routine clinic visit at one of 30 intended study sites.
• Patients who are prescribed rFVIIIFc or rFIXFc, based on the clinical judgement of the treating physician and in discussion with the patient, may be enrolled in the study; the treatment regimens will not be influenced by the study protocol.
• Prophylaxis with rFVIIIFc or rFIXFc will be prescribed according to usual clinical practice and the relevant summary of product characteristics.
• The switch to rFVIIIFc or rFIXFc can occur at any time before the enrolment visit, or at the enrolment visit.
• Patients who discontinue prophylactic treatment with rFVIIIFc or rFIXFc will be withdrawn from the study.
• No visits, examinations or procedures apart from those occurring as part of the patient’s routine clinical care are mandated as part of the study.

Patient eligibility and recruitment

• Patient eligibility criteria are shown in Table 2.

Primary objective

To describe the real-world use and effectiveness of rFVIIIFc and rFIXFc in patients with haemophilia A or B in Germany.

Secondary objectives

• To describe the real-world use and effectiveness of rFVIIIFc and rFIXFc compared with previous non-Fc factor replacement therapy assessed retrospectively from the first injection with rFVIIIFc or rFIXFc.
• To describe the health economic parameters in patients treated with rFVIIIFc or rFIXFc.
• To evaluate the effectiveness of rFVIIIFc and rFIXFc on health economic parameters compared with previous non-Fc factor replacement therapy assessed retrospecively for patients initiating rFVIIIFc or rFIXFc at enrolment.

The first patient was enrolled in May 2017 and it is expected that the last patient will be enrolled in June 2018 (Figure 2).

Primary endpoints

• The endpoints in PREVENT are shown in Table 3.

Data collection

• Clinical and patient-reported outcomes will be collected at routine clinical care visits during the 24-month prospective phase.

• As well as information relating to patient demographics and haemophilia history, data collected at the enrolment visit will include information about the prophylactic factor product prescribed and dispensed in the 12 months before the first injection with rFVIIIFc or rFIXFc, and missed planned activity or productivity because of haemophilia in the previous 3 months.

• Data relating to adverse events leading to permanent discontinuation of rFVIIIFc or rFIXFc, or serious adverse events occurring during treatment with rFVIIIFc or rFIXFc, will also be collected.

Statistical methods and sample size

• Descriptive statistics will be used and no formal statistical hypothesis testing is planned.
• The enrolment target is 75 patients with haemophilia A and 25 patients with haemophilia B; a minimum, 65 patients with haemophilia A and 20 patients with haemophilia B should be enrolled.

CONCLUSIONS

• PREVENT is an ongoing, prospective, non-interventional study that will generate data on the real-world use and effectiveness of rFVIIIFc and rFIXFc prophylaxis in patients with haemophilia A or B in Germany, as well as providing insights into the rationale for initiating prophylactic treatment with rFVIIIFc and rFIXFc.

STUDY METHODS

Overview of study design

• PREVENT is a 24-month prospective, non-interventional, multicentre, Phase 4 study being conducted in Germany in patients with haemophilia A or B (Figure 1).

• All patients who are currently taking rFVIIIFc or rFIXFc will be recruited to the study.

Table 1: Objectives in the PREVENT study

<table>
<thead>
<tr>
<th>Objective</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Primary objective</td>
<td>To describe the real-world use and effectiveness of rFVIIIFc and rFIXFc treatment over a 24-month period</td>
</tr>
<tr>
<td>Secondary objectives</td>
<td>Over a 24-month period: To describe the real-world use and effectiveness of rFVIIIFc and rFIXFc and their treatment according to the reason for the switch to rFVIIIFc or rFIXFc; To evaluate the clinician and patient satisfaction with rFVIIIFc and rFIXFc treatment according to the reason for the switch; To describe the real-world use and effectiveness of rFVIIIFc and rFIXFc compared with previous non-Fc factor replacement therapy assessed retrospectively from the first injection with rFVIIIFc or rFIXFc; To describe the health economic parameters in patients treated with rFVIIIFc or rFIXFc; To evaluate the effectiveness of rFVIIIFc and rFIXFc on health economic parameters compared with previous non-Fc factor replacement therapy assessed retrospectively for patients initiating rFVIIIFc or rFIXFc at enrolment</td>
</tr>
</tbody>
</table>

Figure 2: PREVENT study timeline

The target is to enrol 100 patients in the study, with an expected 75 patients with haemophilia A enrolled in the rFVIIIFc group, and an expected 25 patients with haemophilia B enrolled in the rFIXFc group (i.e. 3:1 ratio), reflecting the ratio of haemophilia A and B patients in Germany.

Figure 3: PREVENT study sites

Study status

• As of January 2018, 18 patients have been enrolled in PREVENT.
• Out of an intended total of 30 sites (Figure 3), 13 sites have been activated and 7 of these are currently recruiting patients.

Table 3: Endpoints in the PREVENT study

<table>
<thead>
<tr>
<th>Endpoint</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Change in missed planned work or school days</td>
<td>Change between the 24-month prospective period and the 3-month retrospective period</td>
</tr>
<tr>
<td>Health economics endpoints</td>
<td>Change between the 24-month prospective period and the 3-month retrospective period</td>
</tr>
</tbody>
</table>

REFERENCES


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DISCLOSURES

A Table: Grants and personal fees for lectures and consultancy from Alnylam, Bayer, Bayer-BioNTech, Bioverativ, Biogen, Biotest, Boehringer Ingelheim, CSL Behring, DAIICHI SANKYO, CSL Behring, Dyax, EMD Serono, Eloctate, Fisons, Ferrer, Fibretech, Ferring, Ferrer, Genzyme, Gilead, GlaxoSmithKline, Immune Tolerance Network, Innate Pharma, Kephalon, Kogenate, Kyowa Hakko Kirin, LabCorp, Leo Pharma, Lilly, Mérieux, Novo Nordisk, Medtronic, MPM Capital, Octapharma, Ortho, Ortho McNeil, Pfizer, Portola, Plough, Shire, Swedish Orphan Biovitrum, Synthorx, Takeda, Taleo, Thrombosis and Haemostasis Society of North America, Thrombosis Research Institute, Tiana, Tibotec, Travira, UCB, Unichem, the University of Washington, Voyager Therapeutics, Virotta, Wyeth.

B Mitesz: Grants and personal fees for lectures and consultancy from Alnylam, Bayer, Bayer-BioNTech, Biogen, Biotest, Boehringer Ingelheim, CSL Behring, DAIICHI SANKYO, CSL Behring, Dyax, EMD Serono, Eloctate, Fisons, Ferrer, Fibretech, Ferring, Ferrer, Genzyme, Gilead, GlaxoSmithKline, Immune Tolerance Network, Innate Pharma, Kephalon, Kogenate, Kyowa Hakko Kirin, LabCorp, Leo Pharma, Lilly, Mérieux, Novo Nordisk, Medtronic, MPM Capital, Octapharma, Ortho, Ortho McNeil, Pfizer, Portola, Plough, Shire, Swedish Orphan Biovitrum, Synthorx, Takeda, Taleo, Thrombosis and Haemostasis Society of North America, Thrombosis Research Institute, Tiana, Tibotec, Travira, UCB, Unichem, the University of Washington, Voyager Therapeutics, Virotta, Wyeth.