**INTRODUCTION**

**OBJECTIVE**

The objective of this study was to evaluate the efficacy and safety of BAY 94-9027 for prophylaxis and treatment of bleeds in adolescents and adults with severe hemophilia A.

**METHODS**

Patients and Study Design

- **Phase 3, multicenter, open-label, single-blind, treatment study:** PROTECT VIII Kids, ClinicalTrials.gov identifier: NCT01757686 was conducted at 31 centers in 13 countries from May 2013 to March 2015.
- **Patients:** 10 years to <18 years of age with severe hemophilia A (FVIII < 1%)
- **Inclusion:** Patients were treated with BAY 94-9027 for ≥ 12 months.
- **Exclusion:** Patients were excluded from the study if they had a bleeding disorder other than hemophilia A or had a history of a bleeding disorder.
- **Randomization:** Patients were randomized 1:1 to either a prophylaxis regimen or an on-demand treatment regimen.
- **Study intervention:** BAY 94-9027 was administered subcutaneously to patients in the prophylaxis arm at a dose of 20 or 30 IU/kg once every 7 days.
- **Study population:** The study population consisted of patients who completed the study (n=53).
- **Study endpoint:** The primary endpoint was the percentage of patients with a decrease in the annualized bleeding rate (ABR) for spontaneous bleeds.

**RESULTS**

**Patients**

- **Patients:** 61 patients were treated in the study (Table 1).
- **Study population:** 8 patients did not complete the study due to protocol deviations.

<table>
<thead>
<tr>
<th>Age (y)</th>
<th>Median (range)</th>
<th>Total (n=61)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0–2</td>
<td>0.0 (0; 1.0)</td>
<td>0 (0; 1.0)</td>
</tr>
<tr>
<td>3–5</td>
<td>0.0 (0; 2.0)</td>
<td>0 (0; 1.0)</td>
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<tr>
<td>6–10</td>
<td>0.0 (0; 2.0)</td>
<td>0 (0; 1.0)</td>
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<tr>
<td>11–15</td>
<td>0.0 (0; 2.0)</td>
<td>0 (0; 1.0)</td>
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<tr>
<td>16–18</td>
<td>0.0 (0; 2.0)</td>
<td>0 (0; 1.0)</td>
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**Efficacy**

- **ABR:** The ABR for spontaneous bleeds decreased from 3.0 (1.0; 9.0) to 1.0 (1.0; 3.5) in patients who increased their dosing frequency.
- **FVIII inhibitor development:** No patient developed inhibitors to FVIII following administration of BAY 94-9027.

**Safety**

- **Adverse events:** No major safety concerns were observed during the study.

**CONCLUSIONS**

- **Every-5-days prophylaxis dosing was the most frequently used regimen at the beginning (45%) and end (53%) of the study.
- **In a protocol allowing investigators to tailor prophylaxis treatment to individual patient responses, the long-acting product BAY 94-9027 was effective for prevention and treatment of bleeding in patients aged <12 years with severe hemophilia A once a stable dosing regimen was obtained.
- **No patient developed inhibitors to FVIII following administration of BAY 94-9027.**