Striving for a Bleed Free World – An Interim Analysis from the AHEAD Global & German Studies

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INTRODUCTION

• Repeated bleeding into joints is one of the most serious complications of hemophilia. A more than two to three bleeds into the same joint may cause irreversible and progressive structural damage that compromises health-related quality of life (HRQoL).1,2
• The goal of zero bleeding episodes – or as close to zero as possible – is key to preserving joint health and improving HRQoL in patients with hemophilia.3
• The AHEAD (ADVATE Hemophilia: A Outcome Database) study is designed to assess long-term outcome data in patients with hemophilia A receiving treatment in routine clinical practice.

OBJECTIVE

This study aims to evaluate effectiveness of a prophylactic treatment regimen of ADVATE in severe and moderate (FVIII < 5%) hemophilia A patients in a real world setting.

METHODS

• Study Design:
  - Post-authorisation, prospective, non-interventional, multicenter study
  - Global recruitment target of > 1000 severe to moderate hemophilia A patients
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    - Global recruitment target of > 1000 severe to moderate hemophilia A patients
    - Recruitment will be carried out by investigators from participating centers
    - Hemophilic patients aged ≥ 12 years who are in regular treatment with ADVATE

• Interim analysis:
  - Interim analysis International Study Arm
    - To assess long-term outcome data in patients with hemophilia A receiving treatment in routine clinical practice

• Study objective:
  - To assess long-term outcome data in patients with hemophilia A receiving treatment in routine clinical practice

• Recruitment:
  - Inclusion criteria:
    - Patients aged ≥ 12 years
    - Hemophilic patients in regular treatment with ADVATE
  - Exclusion criteria:
    - Patients with a history of immune tolerance induction (ITI) or plasma exchange treatment

• Data collection:
  - Data will be collected at baseline and annually
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• Statistical analysis:
  - Descriptive statistics will be used to summarize the data
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• Ethical approval:
  - Ethical approval has been obtained from the Institutional Ethics Committee
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• Study population:
  - Study population:
    - In total, 567 patients have been included in the German study arm
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ENROLLMENT STATUS

• Enrollment Update:
  - Study start: June 2010
  - Study end: June 2011
  - International Arm
    - Status as of 30 June 2016: 556 patients (401 in Germany, 155 in the International study arm) enrolled
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RESULTS

• German Study Arm
  - As of April/May 2015, 371 patients have been enrolled in the German study arm.
  - Overall, 243 completed the year 1 and 239 completed the year 2 visits.

• International Study Arm
  - The second interim analysis of September 2015 includes 376 patients from the International study arm of whom 243 completed the year 1 and 254 completed the year 2 visits.

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DISCUSSION & CONCLUSION

• While this analysis shows good effectiveness results in terms of percentage of patients with zero bleeding episodes, further future research should expand the efforts to investigate why more than 50% of patients continued to suffer from bleeding episodes despite prophylactic treatment.

• Enhanced individualization of prophylactic regimens based on patients’ characteristics and individual FVIII response bears great potential to optimize outcome and perhaps allow for a more efficient use of an expensive resource.

ACKNOWLEDGEMENTS


REFERENCES


DISCLOSURES

1. *Author is an employee of Shire (Hemophilia Healthcare S.A. Wilbraham, E.M., 1998). The study was sponsored by Shire, now part of Takeda

Table 1: Bleeding Rates in the First 2 Years of Observation

<table>
<thead>
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<th>n</th>
<th>SD</th>
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<td>&gt; 2 bleeds</td>
<td>12</td>
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<td>2014</td>
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<td>2015</td>
<td>&gt; 2 bleeds</td>
<td>14</td>
<td>6.2</td>
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