

## Patient – Quality-of-Life Results with Hemlibra in Hemophilia A with Inhibitors

This letter contains information you requested about the effect of Hemlibra® (emicizumab-kxwh) on quality of life in people with hemophilia A with factor VIII (factor 8 or FVIII) inhibitors. This letter includes studies with the strongest and most relevant data.

This information is provided only for educational purposes and not for use in treatment decisions. You should talk with your healthcare provider for specific information and advice about your condition, your individual situation, healthcare coverage, and any current or potential treatments.

### Glossary

**Caregiver Burden:** Caregiver burden is the emotional, physical, and financial stressors placed on family members, friends, or other individuals who care for a person with chronic illness.

**Inhibitors:** In hemophilia A, inhibitors are antibodies against infused FVIII clotting proteins. These antibodies make the infused FVIII products not effective.

**On-demand:** On-demand refers to a treatment that is given as needed. For example, when bleeding occurs.

**Prophylaxis:** Also known as “prophy”, it is a treatment given on a regular schedule to prevent bleeds.

**Patient reported outcomes:** Patient reported outcomes are a reflection of the patient’s perspective on the impact of care intervention on quality of life and ability to function.

### What is Hemlibra?

Hemlibra is a medicine that is approved by the Food and Drug Administration (FDA) for prophylaxis in adults and children with hemophilia A, with or without FVIII inhibitors.<sup>1</sup>

### What are the HAVEN 1 and HAVEN 2 studies?

HAVEN 1 and HAVEN 2 studied how safe and how well Hemlibra prophylaxis worked to prevent bleeds in people with hemophilia A with FVIII inhibitors.<sup>2-4</sup> In HAVEN 1, 109 males 12 years and older, with an average age of 28 years, entered the study.<sup>3</sup> In HAVEN 2, 85 children under 12 years old, and 3 adolescents between 12 - 17 years old less than 40 kg. entered the study.<sup>4</sup> Ages ranged from 1 - 15 years old.

### What patient reported outcomes were measured in these trials?

Patient reported outcomes are reported directly from patients about their quality of life and general health.<sup>5,6</sup> In hemophilia A, these measures help to understand productivity, social and emotional well-being, and ability to do daily activities. The questionnaires have been tested and used by hemophilia experts.



HAVEN 1: A hemophilia-specific questionnaire that measures quality of life was used in people 18 years and older, and a shorter version in people 12-17 years old.<sup>2</sup> A second questionnaire that measures general health status (named EQ-5D-5L) was also used. Responses of people who got Hemlibra were compared with those from people who only used on-demand bypassing agents to treat bleeds (no prophylaxis).



HAVEN 2: A shorter version of the hemophilia-specific questionnaire was used to measure quality of life in children 8-11 years of age.<sup>4</sup> A second questionnaire was completed by caregivers of children with hemophilia A with inhibitors to understand the caregiver’s feeling and their viewpoint of the child’s health.

## How did Hemlibra affect quality of life and health status in adults and adolescents in the HAVEN 1 study?

After 25 weeks on study, adults on Hemlibra had better quality of life and health status compared to those who received on-demand bypassing agents (used no prophylaxis).<sup>2</sup> Improvements were seen as early as 5 weeks after starting Hemlibra, and were maintained throughout the study.

Table 1 shows areas addressed in the 2 questionnaires.

Table 1. HAVEN 1 Patient Reported Outcome Questionnaires <sup>2,7</sup>		
Hemophilia-specific Quality-of-Life Questionnaire		
• physical health	• dealing with hemophilia	• family planning
• feelings	• sports and leisure time	• your future
• view of yourself	• relationships/partners	• your treatment
• work and school		
General Health Status Questionnaire		
• ability to move	• ability to do your usual activities	• mood
• pain/discomfort levels	• ability to take care of yourself	(anxiety/depression)



People who took Hemlibra once weekly felt they had less painful swellings, less joint pain, less pain with movement, and less difficulty walking far.<sup>1</sup>

Adolescents on Hemlibra had improved quality of life as early as 5 weeks after starting Hemlibra, which was maintained throughout the study.<sup>1</sup> Improvements were seen in the total score, with the greatest improvements in the areas of “Sports and School” and “Family”.

## How did Hemlibra impact quality of life for the children in HAVEN 2?

In HAVEN 2, children 8-11 years old answered questions on a shorter version of the quality of life questionnaire.<sup>4</sup> After 49 weeks, improved quality of life was reported, especially around improved physical health. Table 2 shows the areas that these children were asked about in the questionnaire.

Table 2. Quality of Life Questionnaire for Children 8-11 years <sup>4</sup>		
Hemophilia-specific Quality-of-Life Questionnaire for Children (short form)		
• physical health	• family	• sports/school
• feeling	• friends	• dealing with hemophilia
• view of yourself	• other persons	• your treatment

## How did Hemlibra impact caregivers of children with hemophilia A with FVIII inhibitors in HAVEN 2?

Caregivers of children participating in HAVEN 2 were asked for their perception of the child's health and to assess the impact on caregiver burden.<sup>4</sup> Examples of the types of questions are<sup>8</sup>:

How worried/afraid caregiver is as a result of child's hemophilia?

Impact of child's hemophilia on family life?

How concerned caregiver is about child's hemophilia and impact on family?

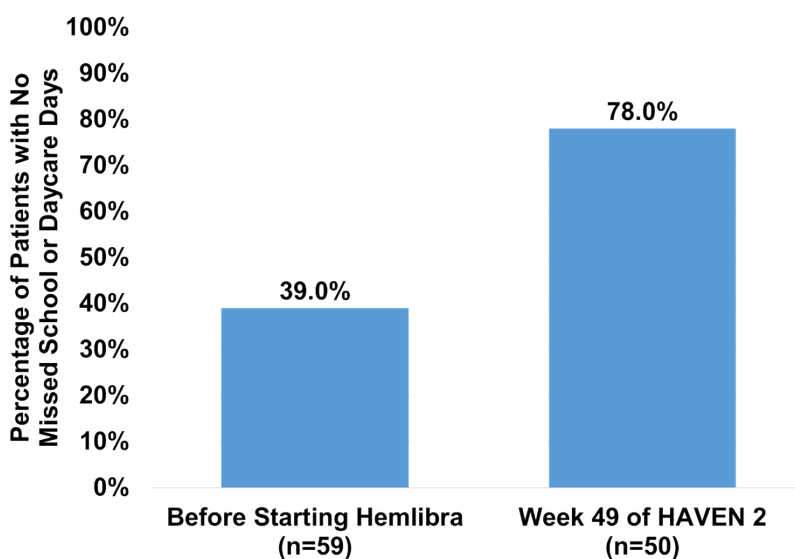
How satisfied caregiver is with child's treatment?

After 49 weeks, caregivers felt their child's health was markedly improved after starting Hemlibra.<sup>4</sup> Marked improvement was also seen in caregiver burden.

## What was the impact of Hemlibra on school attendance?

Out of 59 children from HAVEN 2 who were enrolled in school or daycare, an increase in school or daycare attendance was seen after starting Hemlibra and was maintained through Week 49 of the study (Figure 1).<sup>4</sup>

**Figure 1. Percent of Children with No Missed School/Daycare Days<sup>4</sup>**



### **Patient - Quality-of-Life Results in Hemophilia A with Inhibitors References**

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