



Perceptions, needs and expectations of patients with hemophilia toward gene therapy

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Free paper session: Gene Therapy

Wednesday 24 April, 15:16–15:30

Disclosures

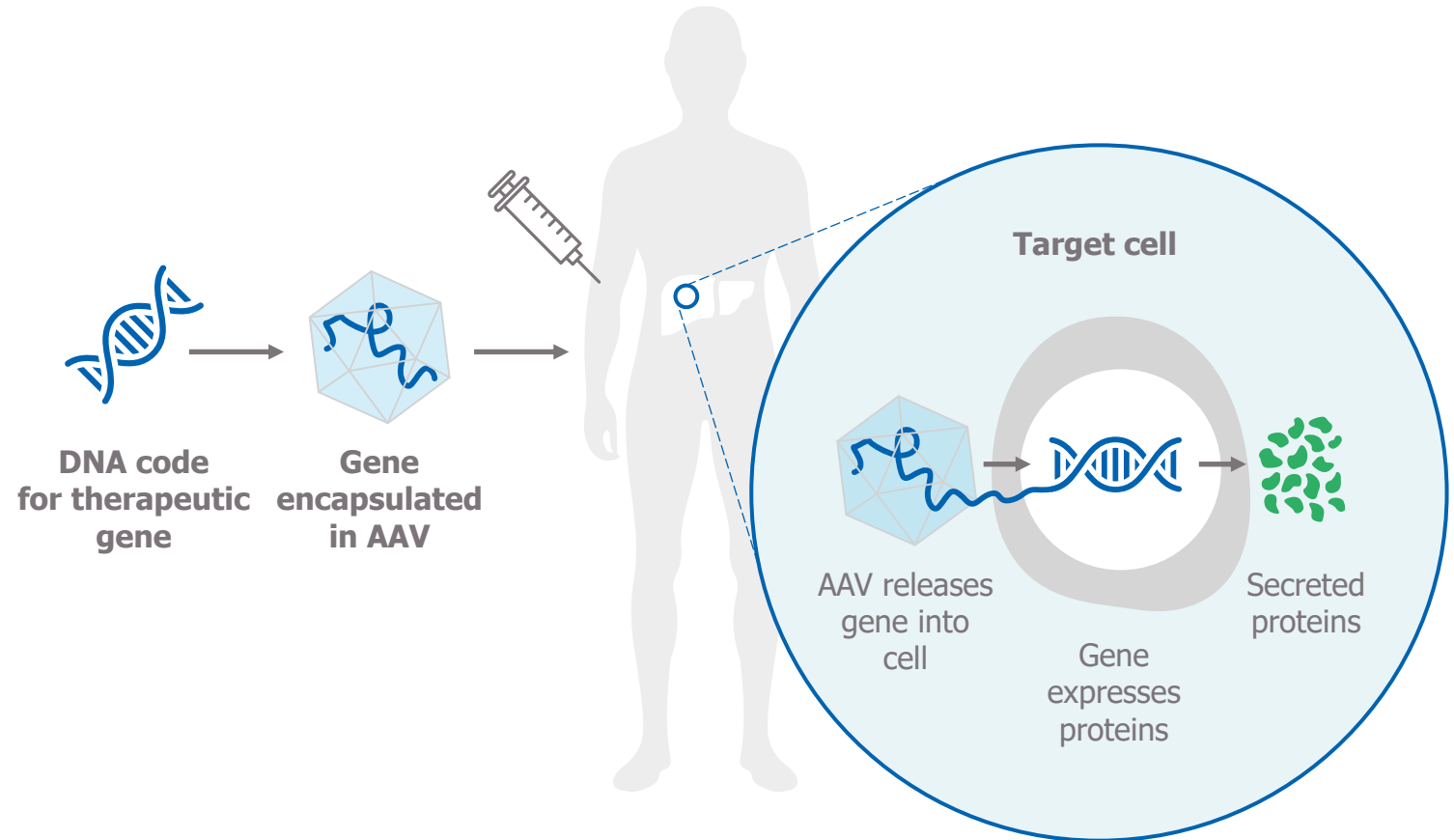
Laurent Frenzel

- Consulting or advisory role: BioMarin, CSL Behring, Pfizer, Roche

Gene therapy for hemophilia

- GT has recently been approved for treatment of adults with severe hemophilia A and B^{1,2}
- GT for hemophilia uses an AAV vector to deliver a functional copy of *F8* (hemophilia A) or *F9* (hemophilia B) to hepatocytes, via a single infusion, to allow long-term expression of FVIII or FIX, respectively^{3,4}
- By removing the need for repeated and lifelong infusions, GT has the potential to improve QoL for adults living with severe hemophilia⁵
- Understanding the patient perspective of GT, a new treatment modality for hemophilia with a novel MoA, is crucial for identifying areas where additional education and support is required

Mechanism of gene therapy for hemophilia⁶



AAV, adeno-associated virus; FVIII, factor VIII; FIX, factor IX; GT, gene therapy; MoA, mechanism of action; QoL, quality of life

1. <https://www.biomarin.com/our-treatments/products/roctavian-valoctocogene-roxaparvovec-rvox/>. Accessed February 2024; 2. <https://www.hemgenix.com/>. Accessed March 2024;

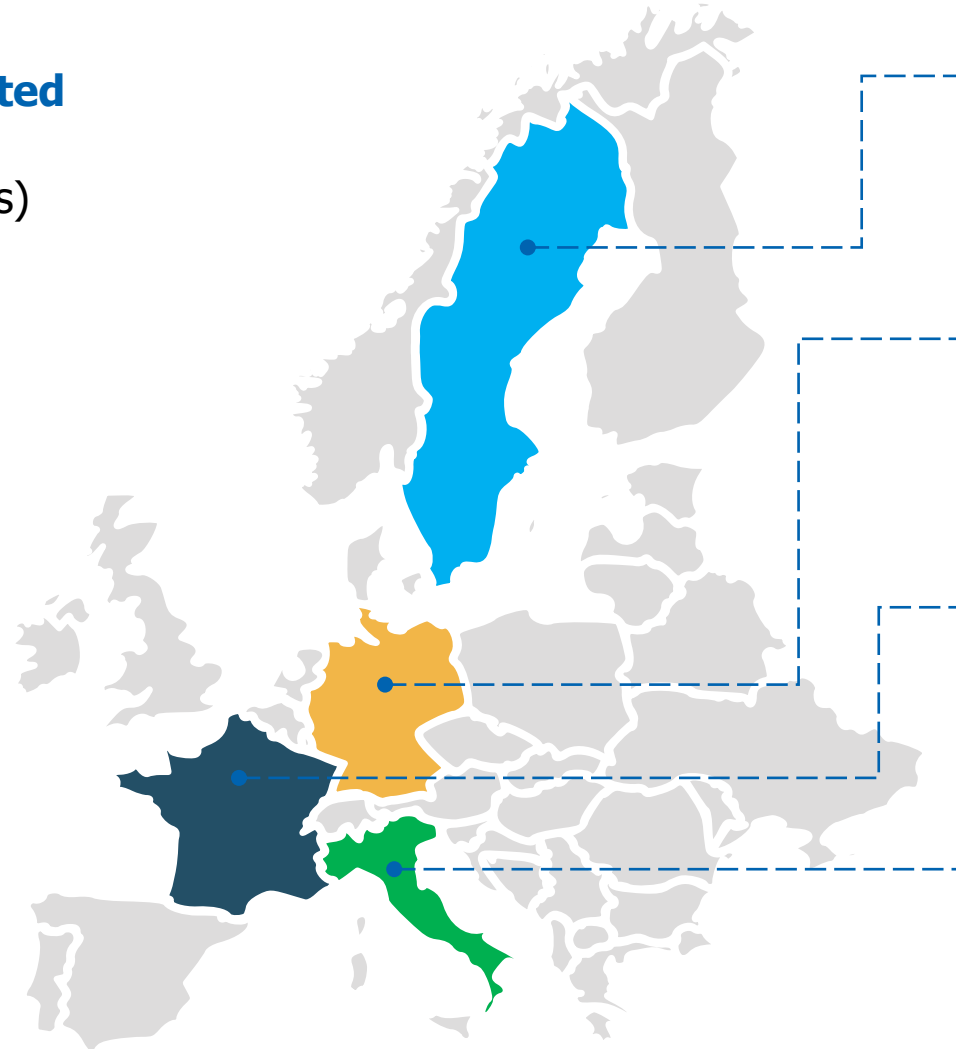
3. Nathwani AC. *Hematology Am Soc Hematol Educ Program* 2022;2022:569–78; 4. Soroka AB *et al. Int J Mol Sci* 2023;24:10766; 5. Rasul E *et al. J Patient Exp* 2023;10:23743735231193573;

6. Adapted from [Gene therapy for haemophilia – Haemnet](#). Accessed March 2024

Methodology and sample composition

A quantitative online survey concerning hemophilia management and GT was conducted

- Participants (n=139) were males (aged ≥ 18 years) diagnosed with severe hemophilia
- The survey was distributed by patient advocacy groups in France, Germany, Italy and Sweden between 16 November 2022 and 10 March 2023
 - Questions were designed to determine the impact of hemophilia on daily life and knowledge and attitudes towards GT for hemophilia treatment
 - Length: 20 minutes to complete
- All results were analyzed descriptively



Sweden
36 patients

Fieldwork conducted from
23 Nov 2022 to 10 Mar 2023



Germany
33 patients

Fieldwork conducted from
23 Nov 2022 to 10 Mar 2023



France
61 patients

Fieldwork conducted from
16 Nov 2022 to 10 Mar 2023



Italy
9 patients

Fieldwork conducted from
30 Nov 2022 to 10 Mar 2023

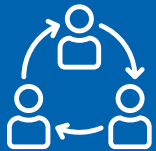
Study objectives



To understand the **perceptions and expectations** of patients with severe hemophilia towards GT



To analyze **patient reactions** to gene therapy and understand **triggers and barriers to use**

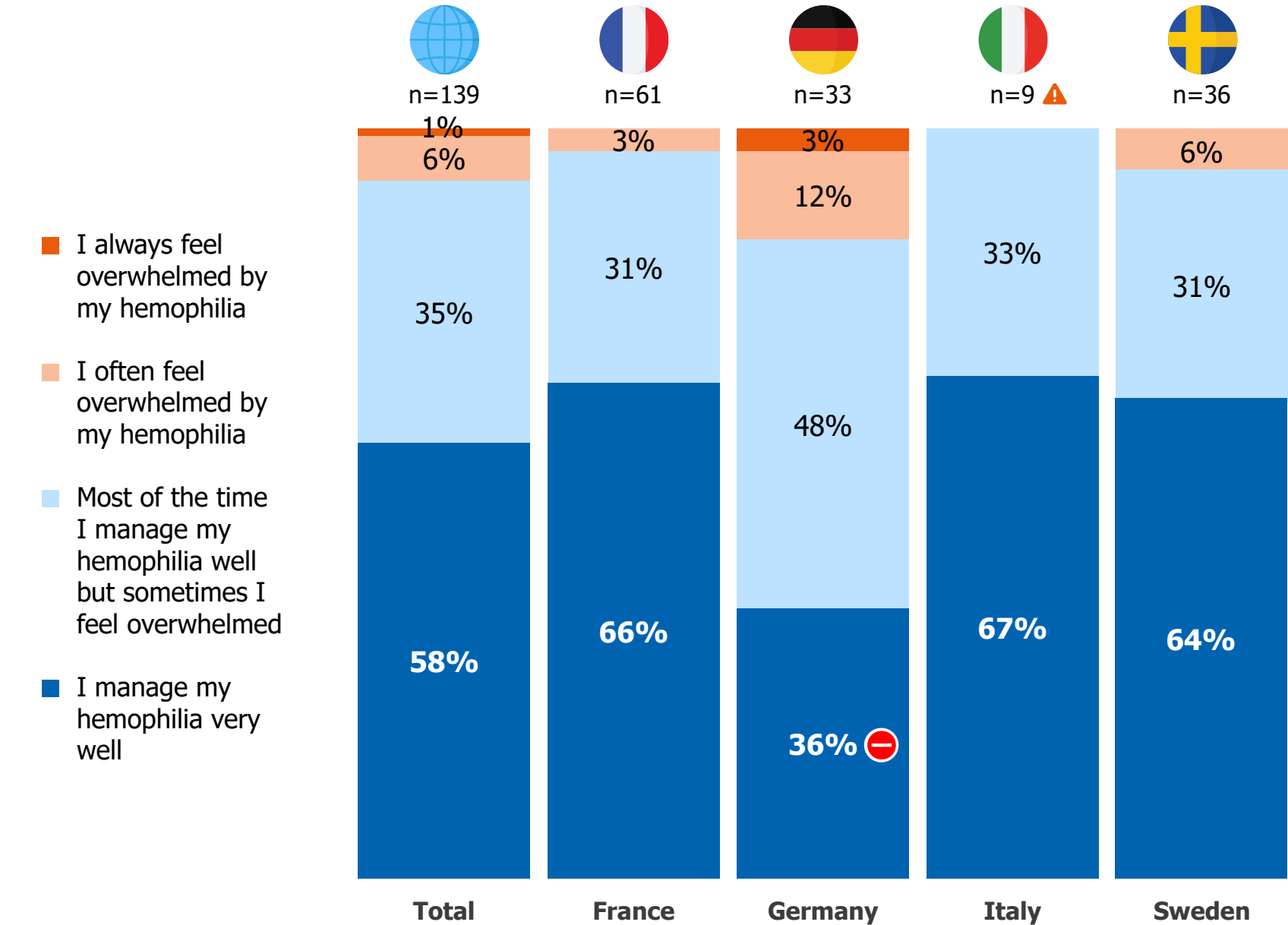


To determine the **necessary support** required for **shared decision making** on GT treatment

Participant demographics

Variable		N=139
Age, n (%)	18–35 years	49 (35)
	36–65 years	83 (60)
	≥66 years	8 (6)
Country, n (%)	France	61 (45)
	Germany	33 (24)
	Italy	9 (7)
	Sweden	36 (27)
Living environment, n (%)	Urban area	67 (48)
	Suburban area	14 (10)
	Small town area	26 (19)
	Rural area	32 (23)
Distance from treatment center, n (%)	<10 km	32 (23)
	10–29 km	17 (12)
	30–49 km	18 (13)
	50–100 km	24 (17)
	100–200 km	19 (14)
	>200 km	29 (21)
Hemophilia type, n (%)	Hemophilia A (severe)	118 (85)
	Hemophilia B (severe)	21 (15)
Developed active inhibitors, n (%)	Yes	24 (17)
Comorbidities (≥5%), n (%)	Hypertension	35 (25)
	Hepatitis	29 (21)
	HIV	24 (17)
	Eczema	8 (6)
	Sleep apnea	8 (6)
	Psoriasis	7 (5)

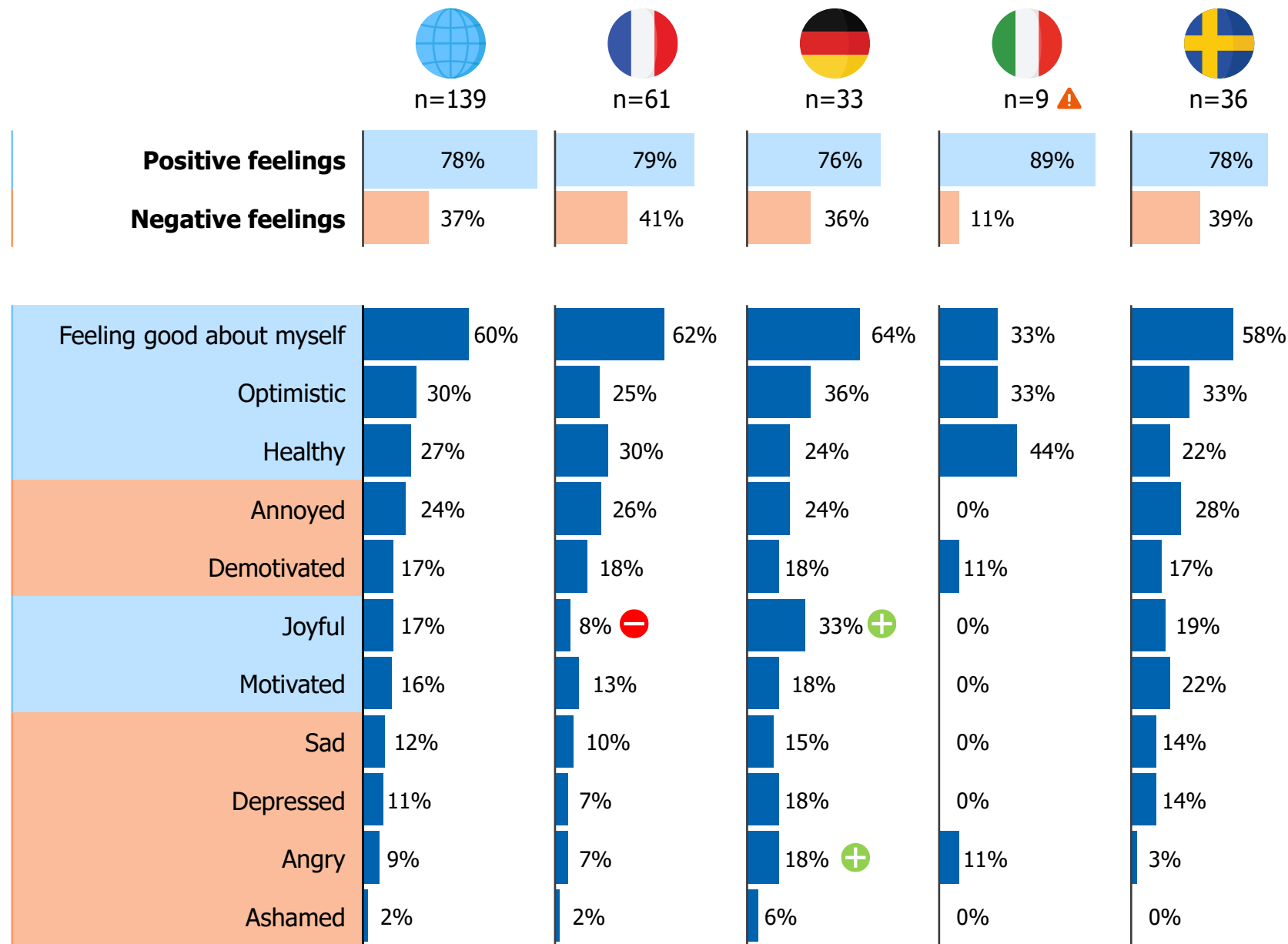
Almost all patients managed well or very well with their hemophilia daily



Base: all respondents / % respondents
 represents a statistically significant lower difference vs other countries
 indicates a low sample size

Q1. Which of the sentences below best describes how you feel about living daily with hemophilia?

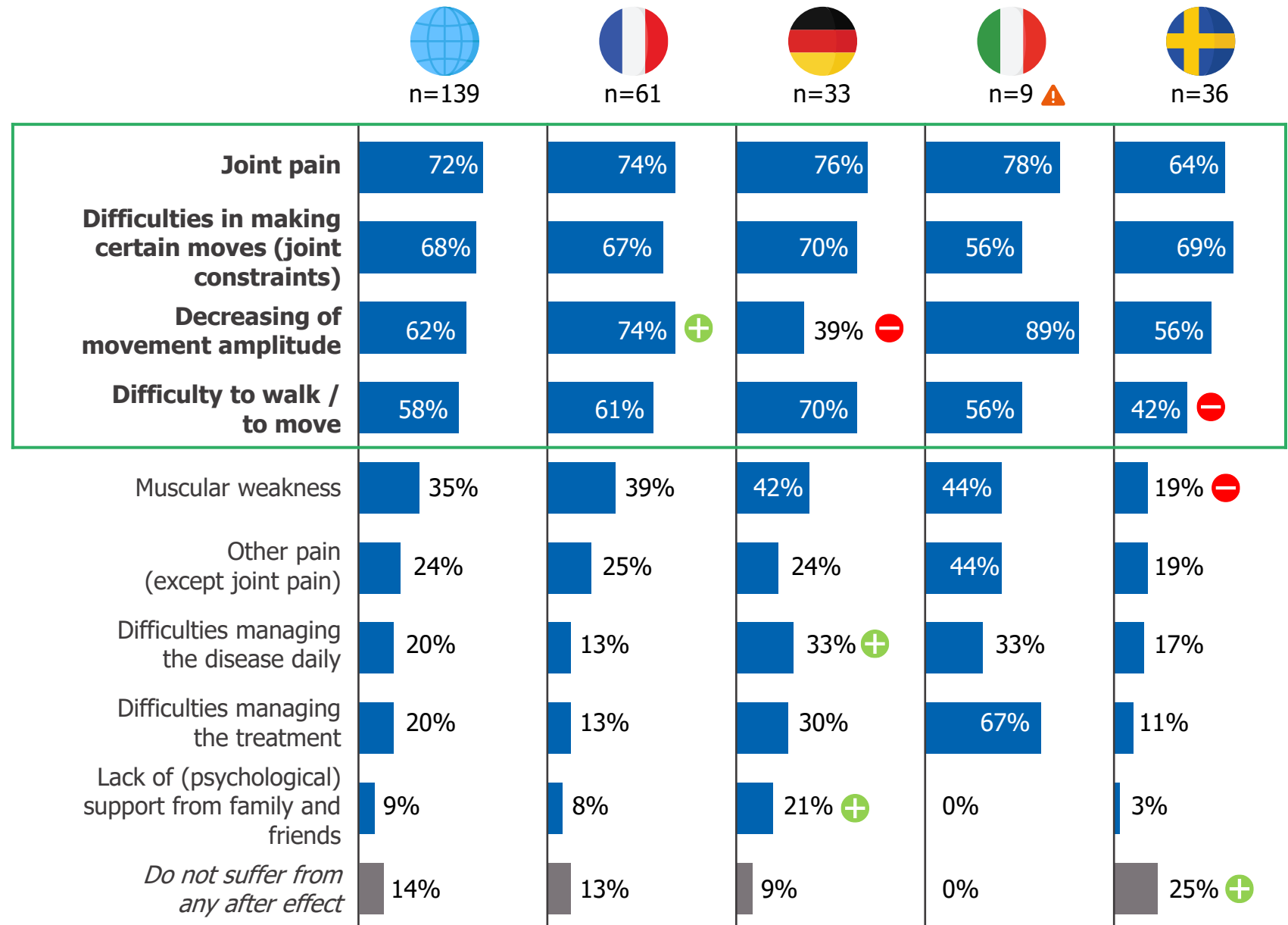
Over the fortnight prior to survey completion, most patients felt positive about their condition



Base: all respondents / % respondents
+ / - represents a statistically significant higher/lower difference vs other countries
! indicates a low sample size

Q3. In the past two weeks, how have you been feeling about your disease?

Joint pain,
joint constraints,
decreasing
movement and
mobility difficulties
had the greatest
burden on daily life



Base: all respondents / % respondents

+ / - represents a statistically significant higher/lower difference vs other countries

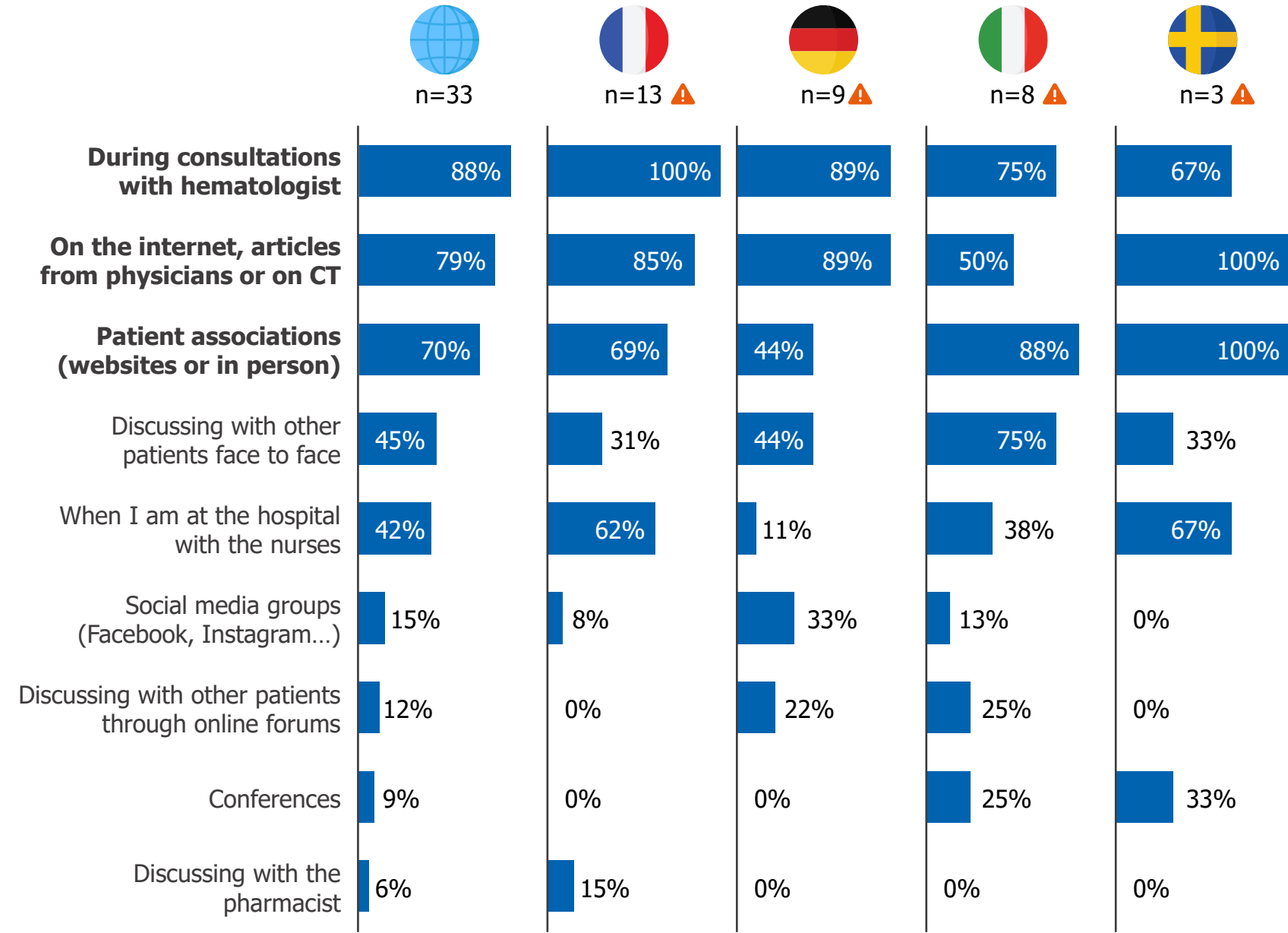
! indicates a low sample size

Q4. Do you suffer from any of the following problems and how do they impact your daily life?

Hematologists,
online medical
articles and patient
associations were
the preferred
sources of
information about
hemophilia and
its treatments

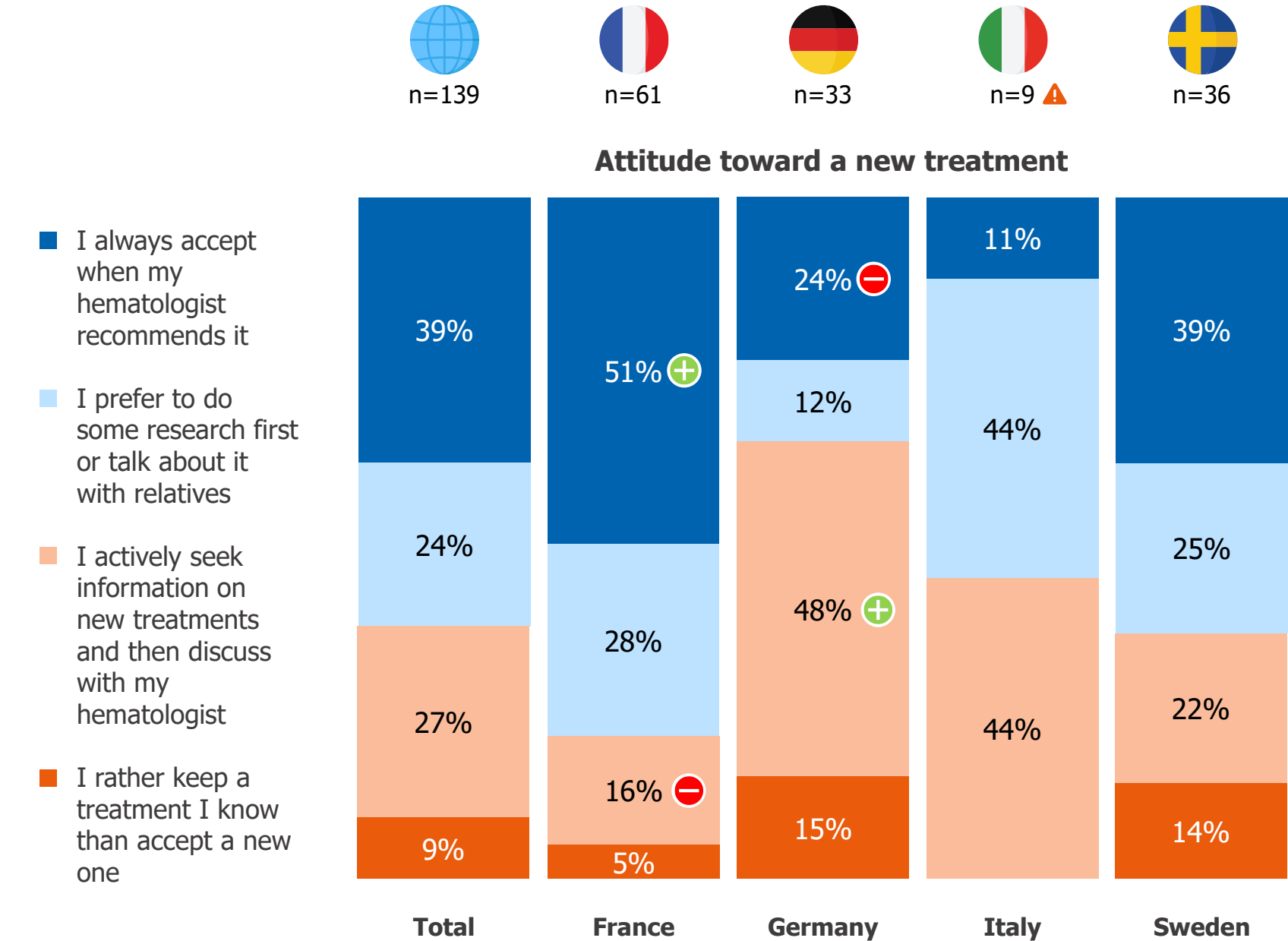
Q11. Which of the following sources do you use the most to get information on hemophilia and/or its treatments?

CT, clinical trial



Base: respondents who regularly search for information about hemophilia and/or its treatments / % respondents
! indicates a low sample size

Approximately 40% of patients would have accepted a new treatment based on their hematologist's recommendation



Q13. Which of the following best describes your reaction to a new treatment?

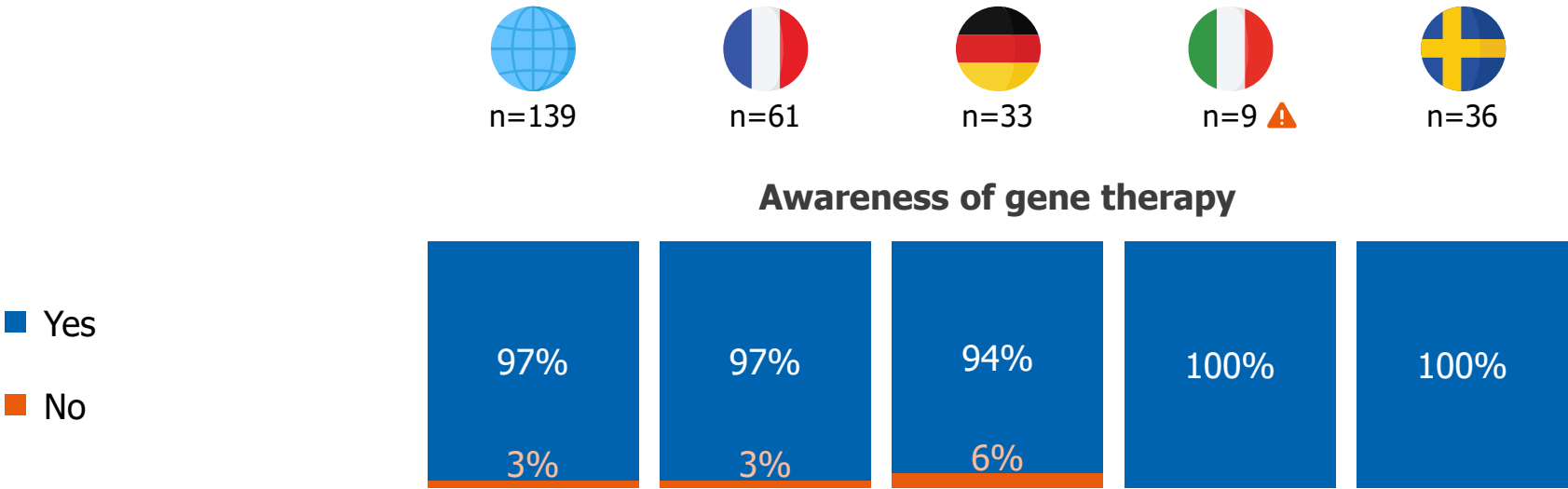
Base: all respondents / % respondents, mean score /10
+ / - represents a statistically significant higher/lower difference vs other countries
⚠ indicates a low sample size

Despite a high level of GT awareness, a significant unmet need for patient education was identified

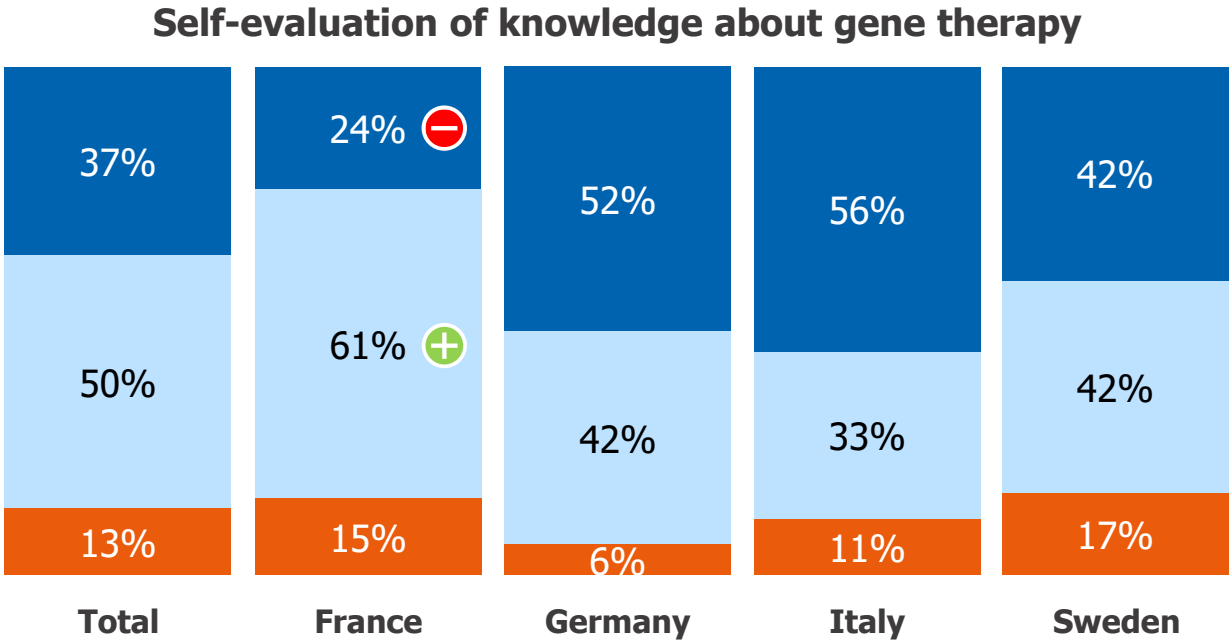
Q15. Have you ever heard of gene therapy for hemophilia treatment?

Q17. Overall, which of the following statements best describes your knowledge of gene therapy for hemophilia?

GT, gene therapy

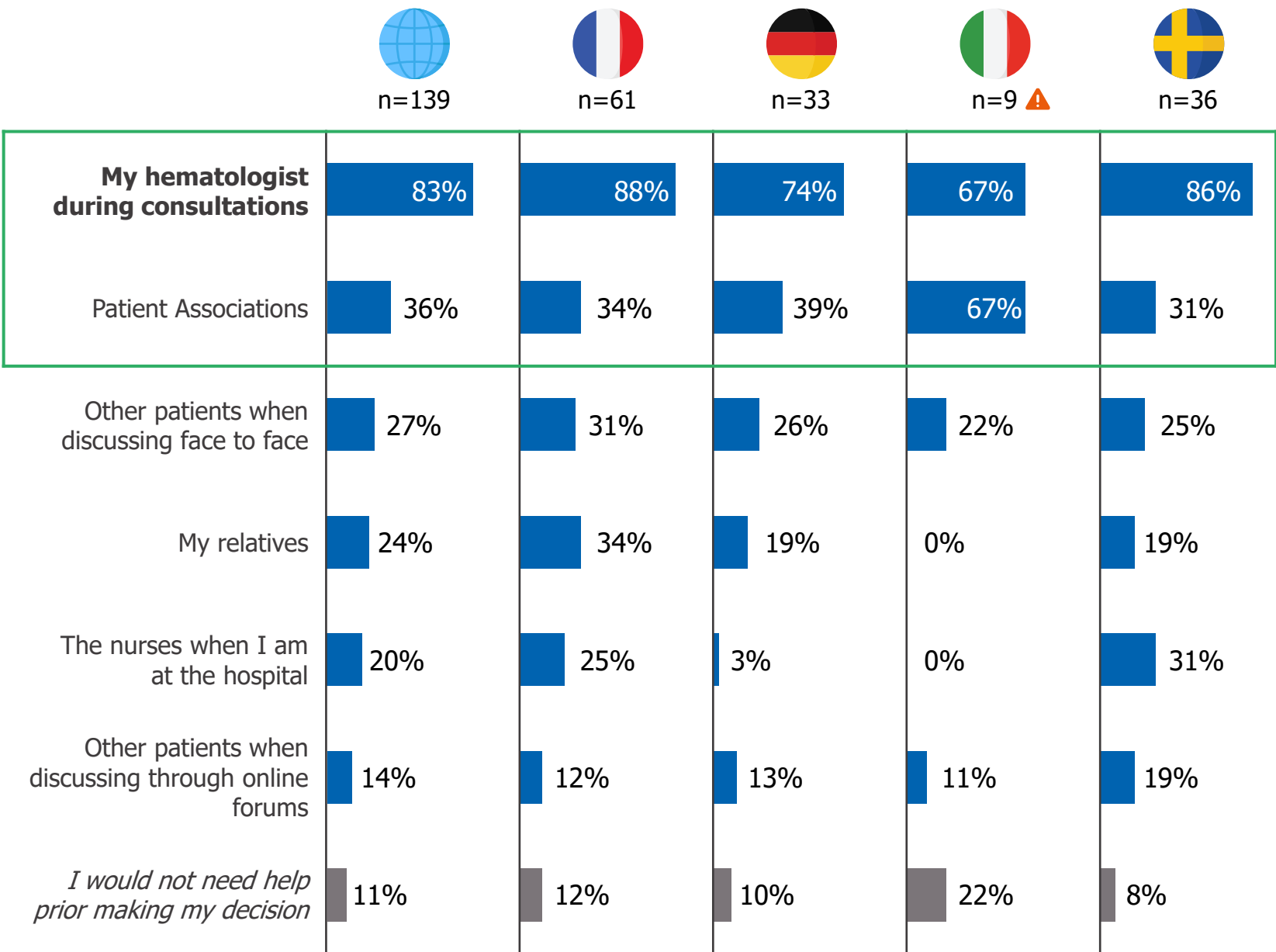


- I know enough about gene therapy to feel comfortable to talk about it with other patients, friends and family
- I've learned about gene therapy, but still don't think that I could really talk about it with someone else
- I do not have a clear understanding of what gene therapy is



Q15 base: all respondents / % respondents; Q17 base: respondents who heard of GT for hemophilia treatment / % respondents
+ / - represents a statistically significant higher/lower difference vs other countries
! indicates a low sample size

Hematologists were the main source of support to aid treatment decision-making



Q26. What would be your preferred source of information and support about gene therapy?

GT, gene therapy

Base: respondents who have heard of GT for hemophilia treatment / % respondents
⚠ indicates a low sample size

Most patients would use GT for hemophilia, even if frequent hospital follow-ups were required

Q18b. Would you be willing to use gene therapy for the treatment of your hemophilia?

Q25. Potential gene therapy might require frequent visits to the hospital for follow-up with the medical team. Based on this information, how likely are you to use a potential gene therapy for hemophilia treatment?

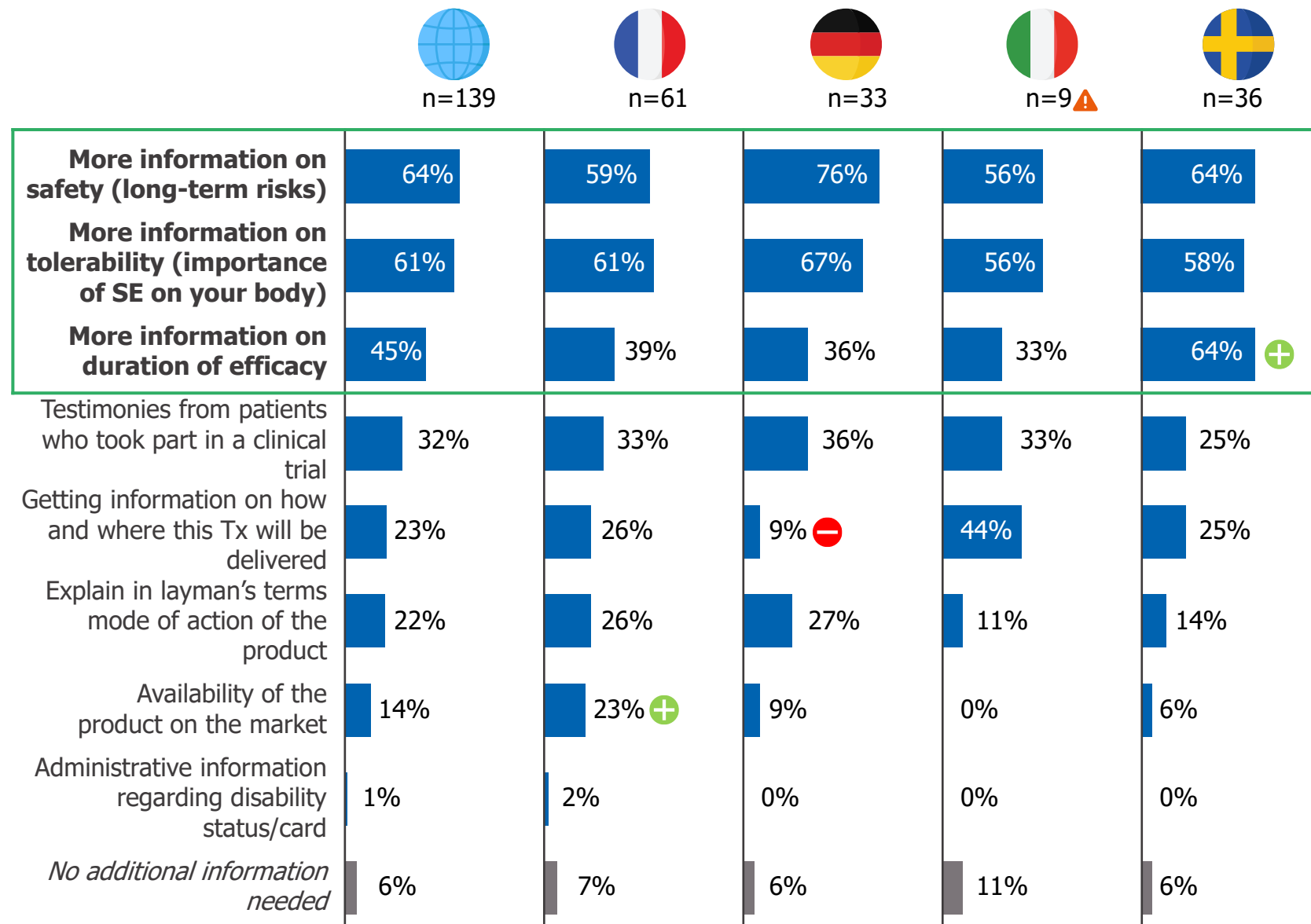
GT, gene therapy

Intention to use gene therapy for hemophilia treatment

Likelihood to use gene therapy considering it might require frequent hospital follow-up							
		Certainly would not use ($<10\%$ chances)	Probably would not use ($10\text{--}30\%$ chances)	Not sure whether would use ($31\text{--}50\%$ chances)	Probably would use ($51\text{--}80\%$ chances)	Certainly would use ($>80\%$ chances)	
	n=	135	20	15 ⚠	26	42	32
	Total	15%	11%	19%	31%	24%	
I would definitely use it	27	20%	1%	0%	0%	4%	15%
I would probably use it	66	49%	0%	1%	13%	27%	8%
I would probably not use it	25	19%	4%	7%	7%	1%	0%
I would definitely not use it	17 ⚠	13%	10%	2%	0%	0%	1%

Base: respondents who have heard of GT for hemophilia treatment / % respondents
⚠ indicates a low sample size

Information on safety and tolerability were top priorities for patients considering GT for hemophilia



Q27. And what additional information would you need in order to consider a potential gene therapy for hemophilia treatment?

GT, gene therapy; pts, patients; SE, side effects; Tx, treatment

Base: all respondents / % respondents

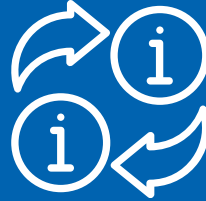
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Conclusions



This study provides valuable insights into the **perspectives and expectations** of adults with severe hemophilia regarding GT



While most were open-minded to GT, an **unmet need** for additional information on this innovative therapy was identified

Information on safety, tolerability and duration of efficacy were the top priorities for patients considering GT for hemophilia

Hematologists were identified as the main source of information about hemophilia and its treatments, and support to aid treatment decision-making



Most patients believed the benefits of GT for hemophilia outweigh the inconvenience of frequent hospital follow-ups

Acknowledgements

- The authors would like to thank the individuals who participated in the study



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