

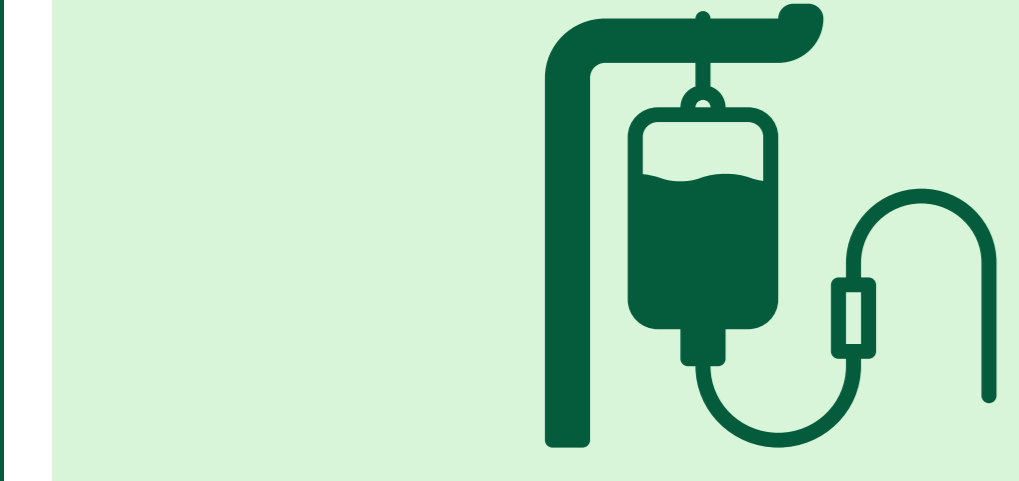
# Improved joint health after gene therapy with dirloctocogene samoparvovec (SPK-8011) in people with hemophilia A

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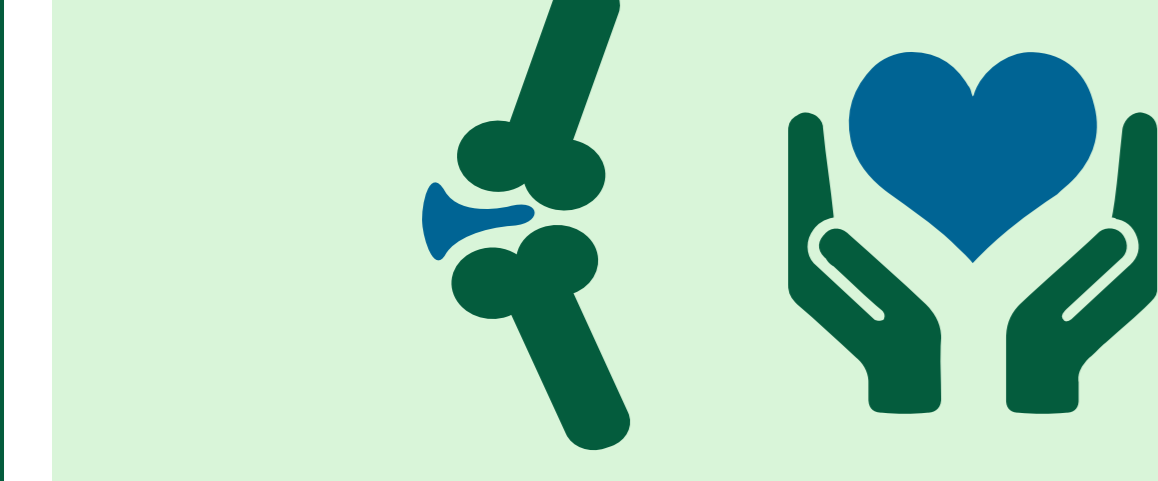
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## Summary and Conclusions

This exploratory analysis investigates the clinical impact on joints for a subset of participants who received the investigational gene therapy dirloctocogene samoparvovec in a Phase I/II trial (NCT03003533/NCT03432520)



Clinically meaningful improvement in total Hemophilia Joint Health Score was observed following dirloctocogene samoparvovec infusion, alongside improvements in self-reported function and target joint resolution



Improvements in joint health were observed in all groups: prior on-demand or prophylactic factor VIII treatment, and presence or absence of target joints at baseline



Although interpretation of these results is limited by the small sample size, exploratory observations support improvement of joint health following treatment with dirloctocogene samoparvovec. Additional investigation is warranted

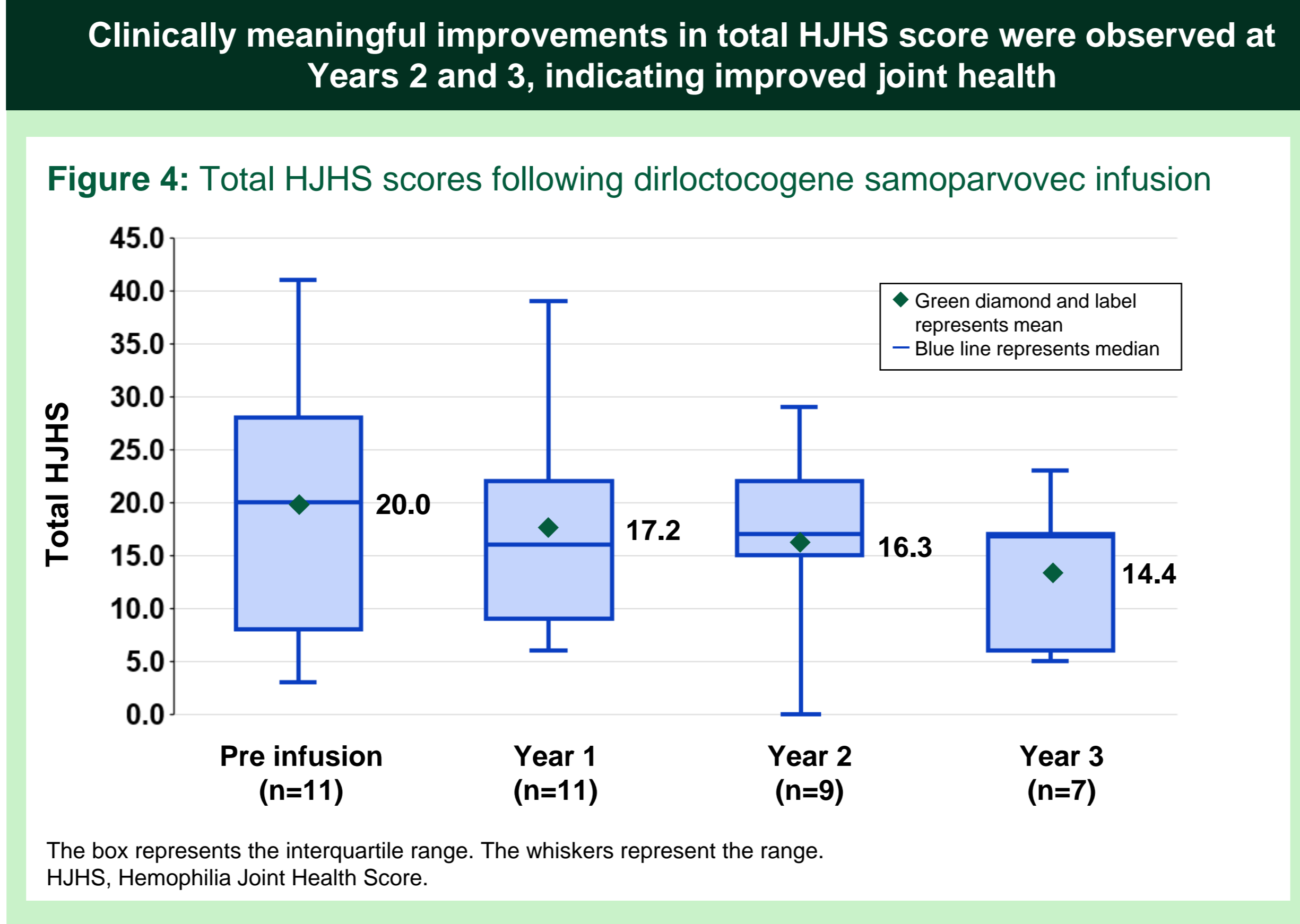
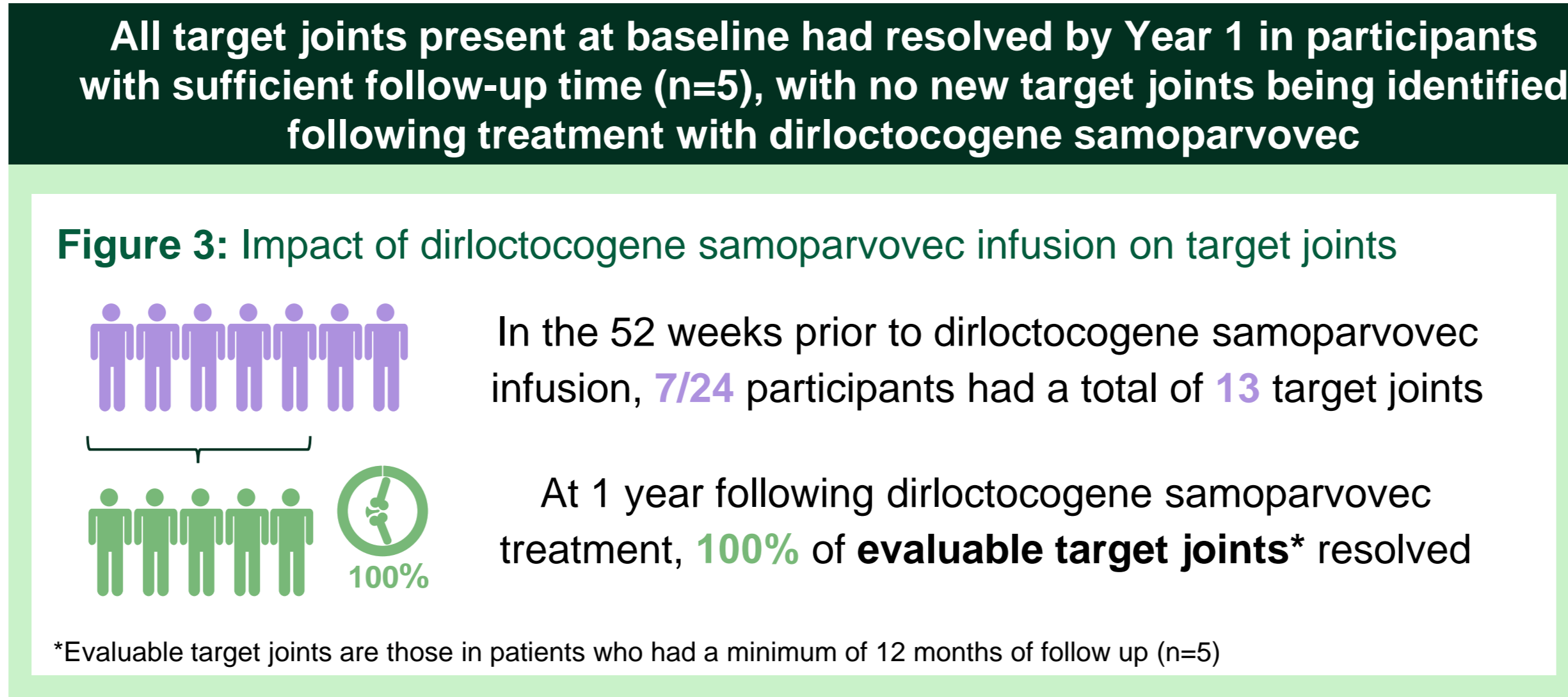
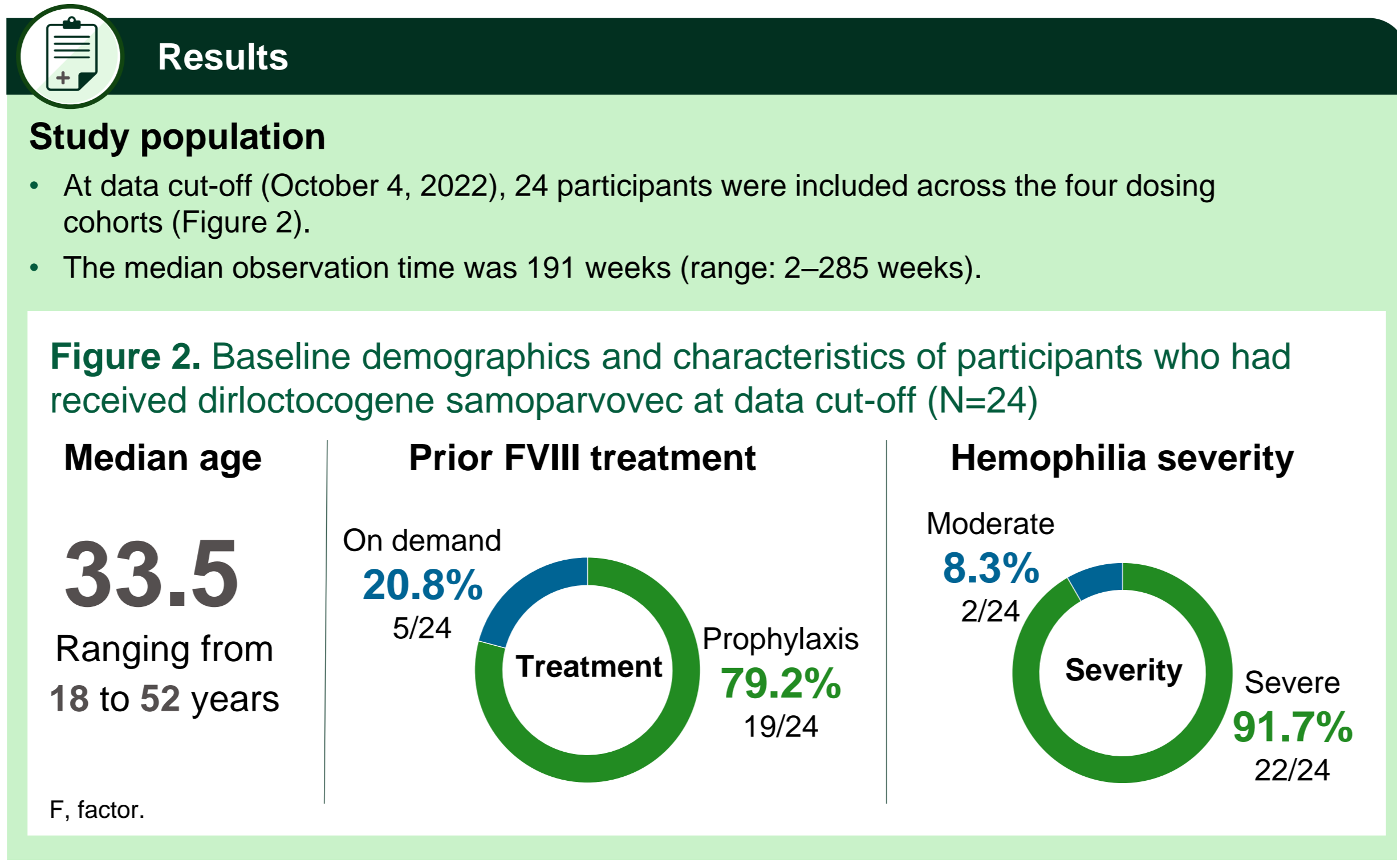
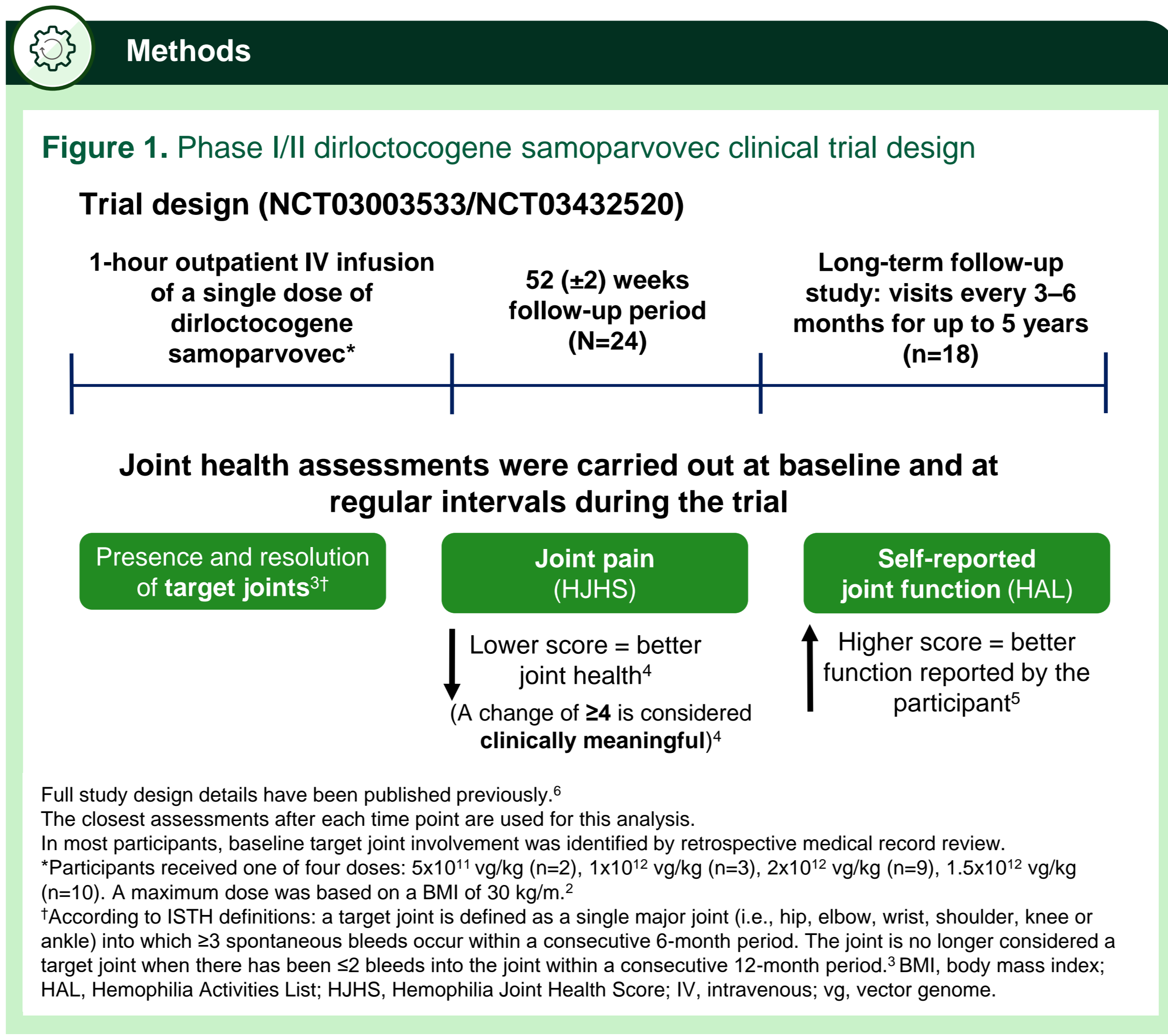


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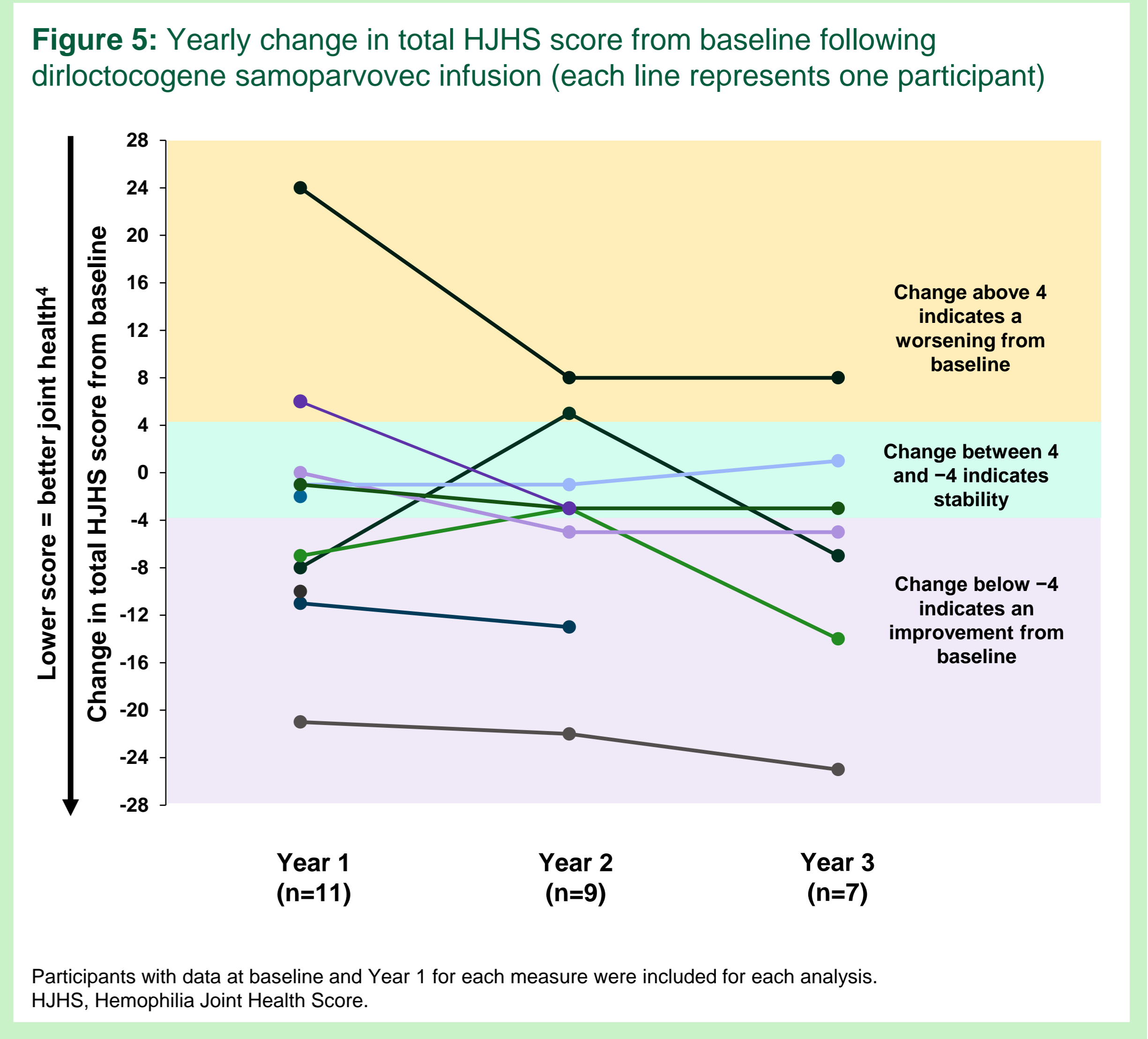
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### Background

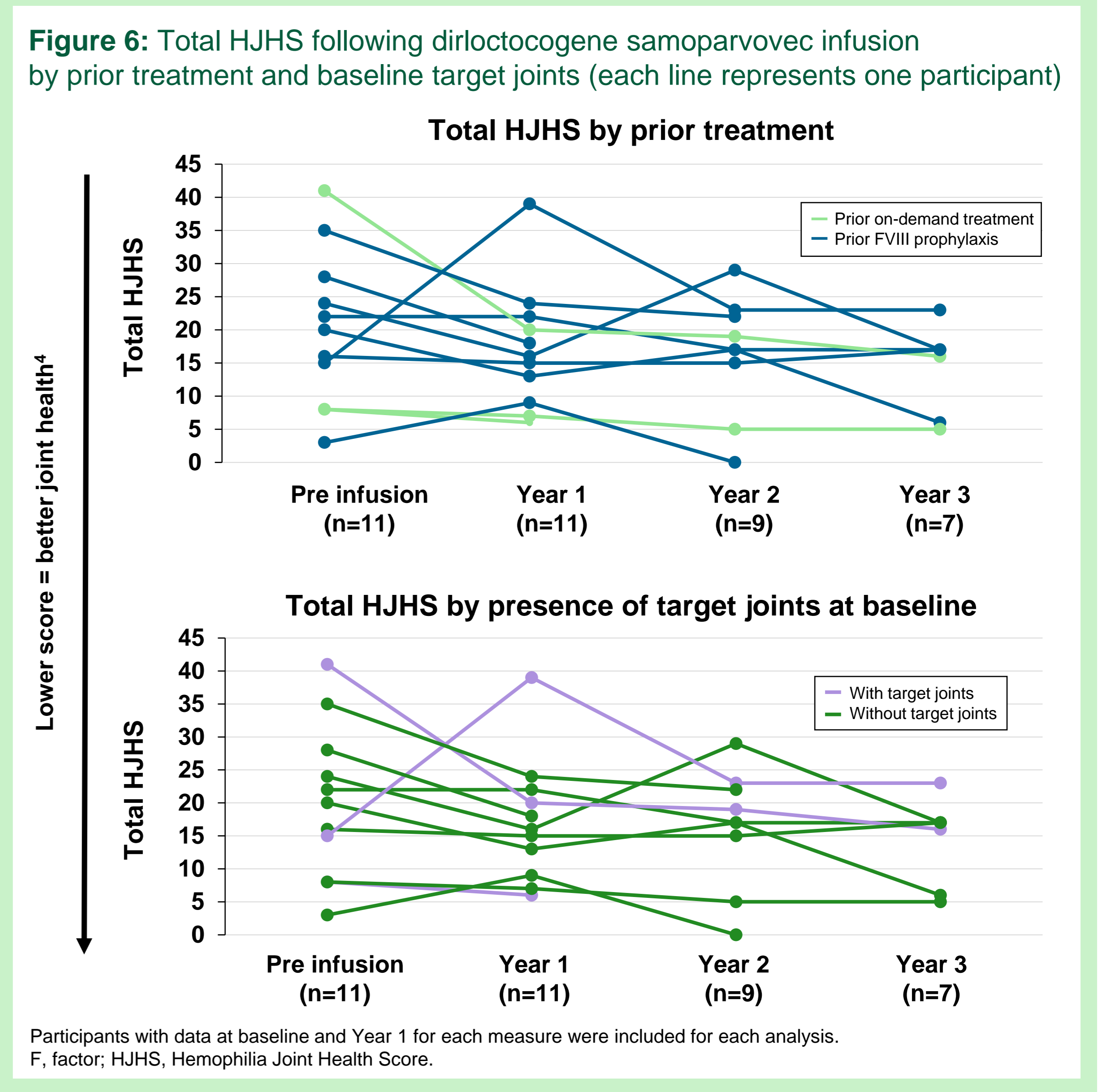
- Hemophilia A (HA) is a bleeding disorder associated with frequent bleeding into the joints. This can cause hemophilic arthropathy, a common complication of hemophilia.<sup>1</sup>
- Dirloctocogene samoparvovec (SPK-8011) is an investigational gene therapy for HA that uses a modified adeno-associated vector to deliver factor (F)VIII to liver cells.
- The phase I/II trial (NCT03003533/NCT03432520) investigating dirloctocogene samoparvovec reported reductions in annualized bleeding rate (ABR) of 99% and 82% for people with HA previously receiving on-demand and prophylactic FVIII treatment, respectively.<sup>2</sup>
- The aim of this analysis is to report the clinical impact of dirloctocogene samoparvovec on the joints of people with HA who participated in the phase I/II trial.



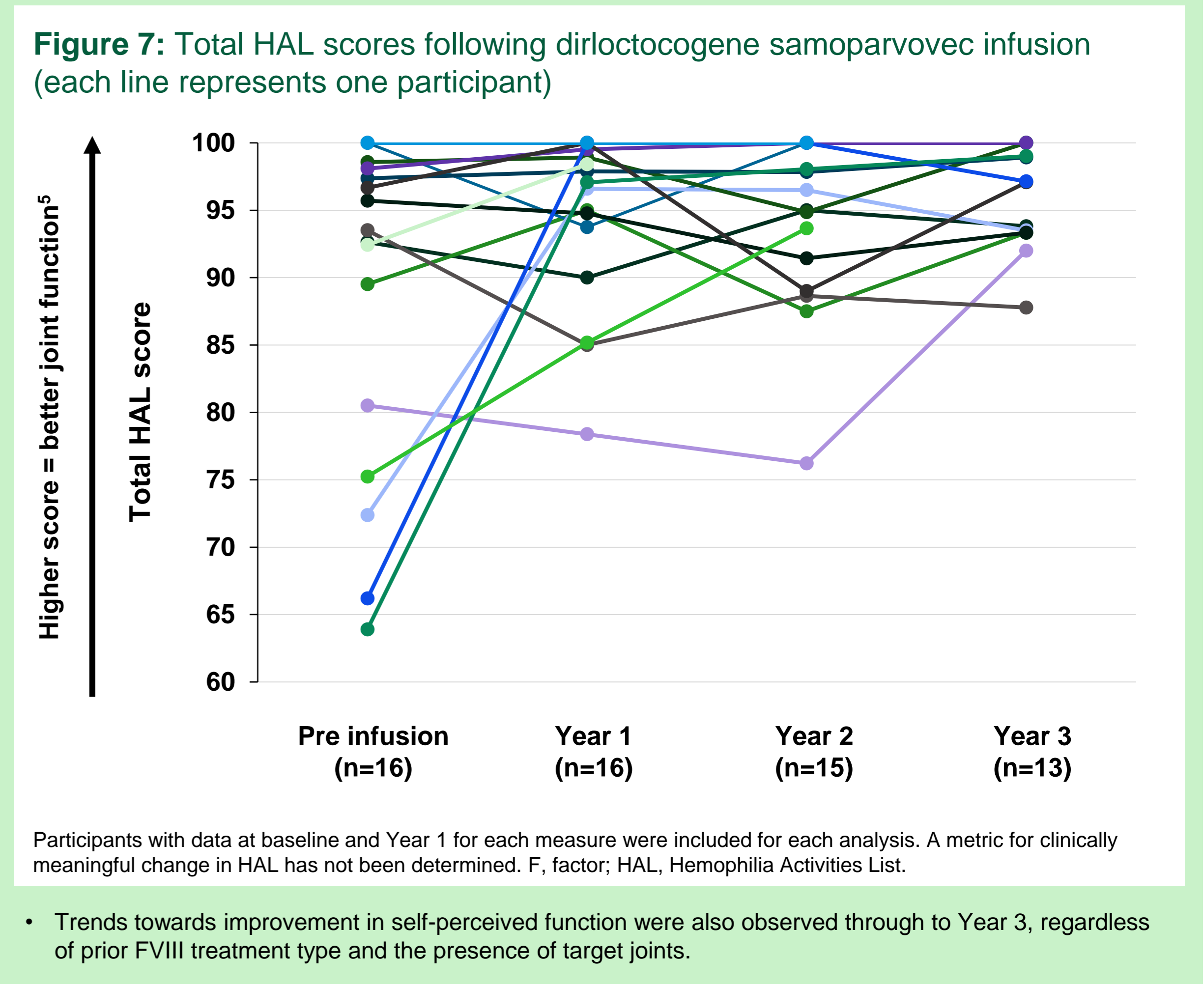
### Clinically meaningful changes in total HJHS score were observed between baseline and Years 2–3 for almost all individual participants



### Trends towards improvement in total HJHS were observed regardless of previous on-demand or prophylactic FVIII treatment, or whether target joints were present at baseline



### Trends towards improvement in total HAL score were observed at Year 1 and maintained over Years 2 and 3, indicating an improvement in the participants' self-reported joint function



Presented at the World Federation of Hemophilia (WFH) 2024 World Congress | April 21–24, 2024 | Madrid, Spain

### References

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### Acknowledgments

The authors would like to thank the study participants and their families, the study investigators, coordinators and nurses, and the Sponsor, Spark Therapeutics, Inc. Third-party medical writing assistance, under the direction of the authors, was provided by Jake Hunter, MSc, of Ashfield MedComms, an Inizio company, and was funded by Spark Therapeutics, Inc.

### Disclosures

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