

Patient perspectives of using PED/PROs as primary versus supportive endpoints in the approval of new drugs and devices

Why clinical trials benefit from patient experiences...

Mattias Van Heetvelde

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Declaration of interest

- **Nothing to disclose**

Background

Patientist

- 28y Fontan
- 7y post-HTx
- PH/HFpEF
- SVT
- TI



- PhD
- Oncogenetics
- Ophthalmogenetics
- Bioinformatics

ESC Patient Forum



Presentation outline

- 1. What is important to patients in terms of efficacy?**
- 2. Why is it important to include PRO/PED as endpoints?**
- 3. How important are PRO/PED as endpoints?**

What is important for patients?



What is important for patients?




Patient Reported Outcomes

Any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else

Why are PROs important?

ORIGINAL ARTICLE

Dapagliflozin in Heart Failure with Mildly Reduced or Preserved Ejection Fraction

Authors: Scott D. Solomon, M.D., John J.V. McMurray, M.D. , Brian Claggett, Ph.D., Rudolf A. de Boer, M.D. , David DeMets, Ph.D., Adrian F. Hernandez, M.D., Silvio E. Inzucchi, M.D.,  [+35](#), for the DELIVER Trial Committees and Investigators[†] [Author Info & Affiliations](#)

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- **Time: 2018-2022**
- **EP: Progression/death**
- **Results: 18% lower risk**
- **PRO: KCCQ**

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- Time: 2018-2022
- EP: Progression/death
- Results: 18% lower risk
- PRO: KCCQ

- Time: 04/2024-07/2024
- ↓ fatigue
- ↓ edema
- ↓ dyspnea
- ↑ activity
- ↑ QoL

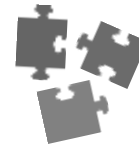
How important are PROs as endpoints?

OPEN ACCESS

ARVO Annual Meeting Abstract | June 2022

Efficacy and safety of seprofarsen, an intravitreal RNA antisense oligonucleotide, for the treatment of CEP290-associated Leber congenital amaurosis (LCA10): a randomized, double-masked, sham-controlled, Phase 3 study (ILLUMINATE)

Primary analysis



Post-hoc analysis



How important are PROs as endpoints?

OPEN ACCESS

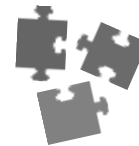
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Patient Experience Data

**“Sepofarsen meant the difference between touching my children’s faces in the morning and seeing them!
How that does not meet *the primary endpoint?!*”**

Primary analysis



Secondary analysis



How important are PROs as endpoints?



PROs > CROs



PROs ~ CROs

Conclusion

- **PROs reflect the patient needs**
- **PROs to be included in (almost) every trial**
- **PROs improve important benefits/insights**
- **PROs have a similar value to CROs, use them as such!**

Background

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