Patient and Carer Feedback on a Nephrology Gene Therapy Study Concept

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Introduction

There is limited experience with gene therapy in nephrology. PS-002 recently entered clinical development and is a gene therapy delivering the complement factor I (CFI) gene to glomerular cells.

Patient input was collected to optimize the design of the phase 1 trial and inform future development of PS-002 and coming gene therapies for renal disorders.

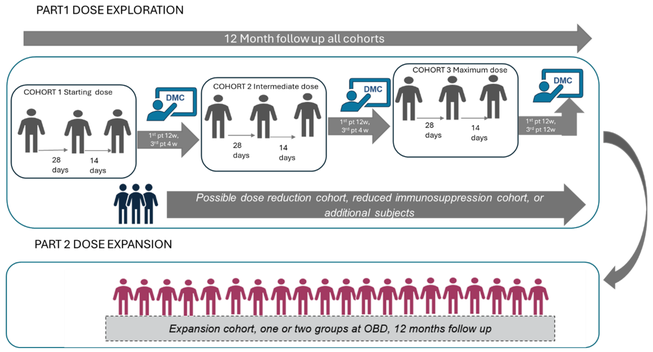
Methods

Virtual insight sessions were conducted with IgA nephropathy patients and patient carers in the US and UK.

Each session involved 6 participants and was conducted by the IgA Nephropathy Foundation in the US and the National Institute for Health and Care Research in the UK.

Participants received advance detailed information on the risks and requirements associated with participation in the PS-002 phase 1/2 gene therapy trial, and feedback was collected and summarized.

PS-002-101 Study Overview

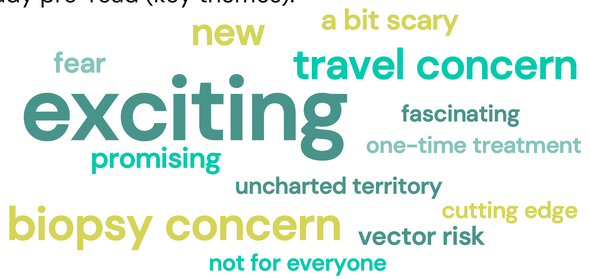


IgAN Patient Reported Symptoms

Most impactful disease symptoms according to IgA Nephropathy patients (UK).

Results

Patients' thoughts on a gene therapy study based on the study pre-read (key themes):



Main points made by patients and carers:

- High interest in a once-and-done therapy for IgA nephropathy
- Concern about participating in a phase 1 trial, but also excitement around trying a new approach
- Patient selection important to maximize chance of benefit
- 4-day inpatient hospitalization acceptable if visitors allowed
- Follow-up biopsy acceptable if informative for the research, but patients wanted careful walk-through of associated risks
- Baseline biopsy not attractive to IgAN patients already diagnosed with recent biopsy

Results

Main points by patients and carers (cont):

- Reimbursement of all costs critical to many patients
- Mixed feedback on corticosteroid course to minimize gene therapy side effects. Patients with prior experience of steroid therapy were the most negative

Conclusions

Patients and carers were cautiously optimistic, with the opportunity for a "once-and-done" therapy and superior efficacy mentioned as main benefits.

Efforts to minimize baseline biopsies, inpatient stay, financial impact and use of corticosteroids are particularly important to patients.

Input on study design & patient facing materials were incorporated into the ongoing clinical study, resulting in a better and more patient friendly trial (see poster INFO15-FR for details).

Generation and publication of robust safety data will be important to alleviate patient worries.

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