Having a strategy/plan that included drug development and access
Engaging early with pharmaceutical companies, the relevant disease community and other key stakeholders such as HTA bodies.
Staff capacity and dedicated time
Accessing or building skills and expertise
Financial resources to support participation

The PAGs valued independent support that was practical rather than theoretical, required a limited time commitment and was able to fit around other demands on staff/volunteers. PAGs also noted a need for ongoing support to refine and implement action plans.
The pilot included a selection process, a readiness assessment, structured workshops, action planning resources and one to one support sessions. At the end of the pilot, all PAGs had a prioritised action plan and reported increased confidence and a better understanding of HTA requirements and how to translate their experiences into evidence. They had identified additional needs around drug development and HTA processes and wanted practical support in this area. They had concerns about whether they would be able to access the financial resources to obtain support and deliver on their priorities.
Recommendations include ensuring the availability of practical support for rare disease PAGS seeking to participate in drug development and access process; providing greater clarity from key stakeholders such as NICE and the ABPI on what funding routes are acceptable for support in this area; and considering a centralised fund that could ensure sustainable and equitable support for all rare disease PAGs.

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ERN-EYE GOOD PRACTICES SERIES: Do's and don'ts on Usher Syndrome

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Background: Based on a collaboration with a representative of an Austrian patient association for Usher Syndrome, the European Reference Network for Rare Eye Diseases (ERN-EYE) launched a leaflet (Fig. 1) aiming to guide healthcare professionals how to welcome and how to communicate with patients with Usher Syndrome at the hospital. This good-practices leaflet was created to address patients' expectations and improve their experience in the network's hospitals but also in all other clinical services (Fig. 2).
Materials and methods: For this project, the ERN-EYE management team worked together with a representative of a patient association and health care experts of the network to gather all important information about Usher Syndrome following a structured approach (Fig. 3). The final version of the leaflet was reviewed and validated by the European Patient Advocacy Group (ePAG) representatives as well as experts of the network.
Results: The leaflet is divided in several parts: clinical manifestation, identified genes, progression of the disease through the years and do's and don'ts. The clear graphic layout makes it easy to find the essential information to retain (Fig. 4).

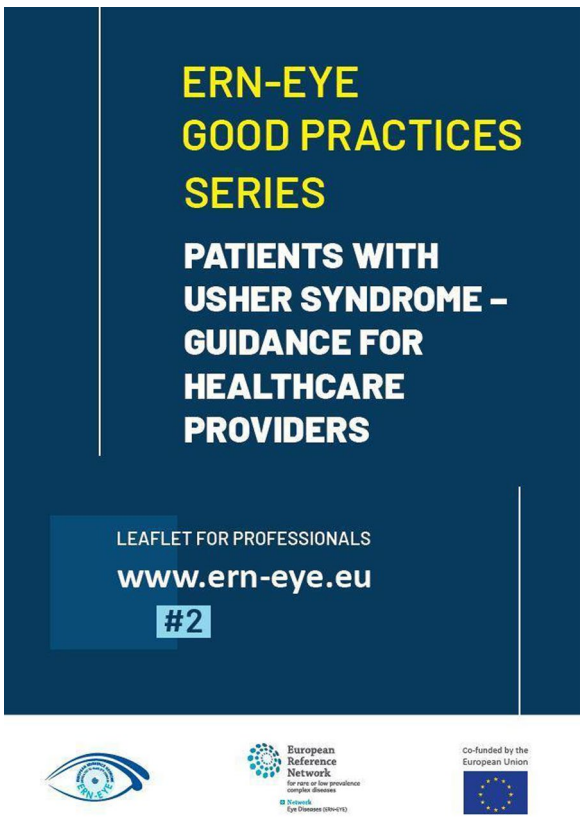

Fig. 1 Cover of the good practices leaflet on Usher Syndrome

Fig. 2 Strategic development of the leaflet: issues and objectives

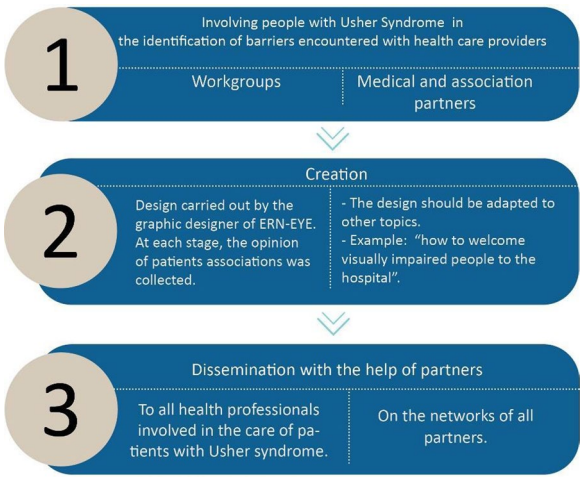

Fig. 3 The project approach was structured around several steps

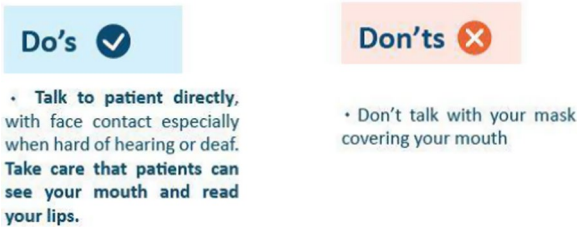

Fig. 4 Example of different do's and don'ts listed in the leaflet

Fig. 25 @5 All partners of the project

Conclusions: The leaflet will be widely distributed within the ERN-EYE network and relevant associations during internal congresses and related conferences. Thus, as it was created thanks to the involvement of patients and healthcare professionals, it fits a real need. It can be translated into several languages as required.
Finally, the project of producing a video associated with the leaflet will enhance dissemination of the information on a wider range.

Acknowledgements
For this project, we wanted to involve users' representatives to fully integrate them in the development of the system so that it reflects their needs and experiences. The partnership was obvious with the ERN-EYE European Patient Advocacy Group (ePAG) representative of an Austrian patient association for Usher Syndrome, as well as with medical experts of the network (Fig. 25 @5). The English text will go through proofreading by our English ePAG representative.

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The H-CARE project: monitoring the healthcare experience of people living with rare diseases

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Aims: The H-CARE project aims to develop a patient feedback mechanism that allows to regularly measure the clinical care experience of people living with rare diseases while ensuring robust, comparable, and independent data collection and results, across the 6000 + rare diseases and across countries.
Background: A pilot survey tested the feasibility of a patient feedback mechanism and of measuring rare disease patients' and carers' experience of clinical care. Because no Patient Reported Experience Measure (PREM) has been developed specifically for rare diseases, this pilot used the PACIC questionnaire [1] for common chronic diseases. It was disseminated from December 2019 to March 2020, to 3905 respondents in 65 countries and 23 languages, living with 900 + rare diseases. This pilot shows that rare disease patients give their clinical care experience a medium–low score (2.5 on a scale from 1 to 5) and seem to have a worse experience of health care than patients with chronic diseases [2]. It also shows that the PACIC questionnaire does not encompass all the aspects of clinical care that are specific to rare diseases, such as emotional support, genetic counselling, coordination between local care and expert centres or coordination between social and medical care.
Method: Based on the results of the pilot survey, the H-CARE project includes the development and validation of two PREMs, one for rare disease patients and one for family members caring for rare disease patients. A scoping literature review is being conducted that, together with upcoming focus groups, will lay the foundation for the definition of a theoretical model of high-quality experience of clinical care for rare diseases that will be based on patients' needs. This theoretical model will then allow to design two questionnaires in several languages, before validating them.
Discussion: Our goal is that by 2026, the two PREMs could be used to measure the clinical care experience of people living with rare diseases as part of a regular patient feedback mechanism, and to monitor the patient centricity of the 24 European Reference Networks (ERNs) that bring together experts from across Europe to ensure that people living with rare and complex diseases can benefit from the best treatment and advice available for their condition.

Acknowledgements
The H-CARE project was initiated by ERN ErkNET (Franz Schaeffer, Claudia Sproedt, Vera Cornelius, Giulia Bassanese), ERN eUROGEN (Wout Feitz, Michelle Battye, Dalia Aminoff), ERN Genturis (Ignacio Blanco, Nicola Reents) and ERN LUNG (Thomas Wagner, Hilde de Keyser, Bernhard Rindlisbacher, Keerthana Iyer). It is supported by Rare Barometer, a EURORDIS-Rare Diseases Europe initiative (Sandra Courbier, Jessie Dubief, Ines Hernando, Matt Bolz-Johnson). The H-CARE Scientific Working Group also includes Prof. Dr. Michel Wensing (Heidelberg University Hospital), Prof. Dr. Elena Levtchenko (Katholieke Universiteit Leuven), Prof. Augustina Jankauskienne (Vilnius University Hospital Santaros Klinikos), Dr. Benoît Arnoult (ICON-PLC) and Dr. Samantha Anthony (SickKids). Emily White (ENDO-ERN) contributed to the scoping literature review.

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VASCA Magazine: a tangible result from the 5-year collaboration of Patient Advocates with the clinicians of the Vascular Anomaly (VASCA) Working Group of VASCERN

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