



# SETTING STANDARDS OF CARE QUALITY!



## Results of the H-CARE Pilot Survey for the development of a validated scale and of a Common Feedback Mechanism to measure healthcare experience for rare diseases in Europe

January 2021

European Reference Networks (ERNs) bring together experts from across the European Union to ensure that people living with rare or complex diseases can benefit from the best treatment and advice available for their condition. ERNs ErkNet, eUROGEN, Genturis and LUNG with the support of

Rare Barometer, have set up the H-CARE Pilot Survey to test the development of a Common Feedback Mechanism that would allow to take into account patients' and carers (family members...) experience with the care delivered by healthcare providers when evaluating the 24 ERNs.



### 1,319 PATIENTS AND CARERS

assessed European healthcare providers treating rare diseases affecting kidneys, lungs, the urogenital area and genetic tumour risk syndromes



### CARE EXPERIENCE IS BETTER

with healthcare providers that are part of a European Reference Network than with other healthcare providers treating the same disease areas

## WITHIN THIS REPORT YOU WILL FIND:

1



A description of the validated questionnaire used in the H-CARE Pilot Survey and how it could be improved to measure experience and satisfaction with care in the 24 ERNs

2



Key results on healthcare experience within European healthcare providers treating rare diseases affecting kidneys, lungs, the urogenital area and genetic tumour risk syndromes

3



Insights on how to implement a Common Feedback Mechanism on patients' and carers' experience with care across the 8000+ rare diseases, across the 24 ERNs and across Member States

This is an initiative of:



Genetic Tumour Risk Syndromes (ERN GENTURIS)



ERN-LUNG  
European Reference Network  
For rare respiratory diseases



eUROGEN  
European Reference Network  
Urogenital Diseases

Supported by:



# 1 USING A VALIDATED QUESTIONNAIRE

A Topic Expert Committee composed of patient representatives, clinicians and managers from ERNs ErkNet, eUROGEN, Genturis and LUNG decided to use a validated questionnaire to measure experience with care in the H-CARE Pilot Survey.

**Validated questionnaires** are used to measure qualitative and subjective concepts, such as anxiety or care experience, through a **fixed series of questions** (or items) that are all related to the concept being measured and that are **combined to give a numerical score**. That is why validated questionnaires are also called **validated scales**.

Because there is no validated questionnaire to measure experience with care for rare and complex diseases, the H-CARE Survey Topic Expert Committee decided to use the **PACIC-S**, which is the short form of the Patient Assessment of Care for Chronic Conditions (or PACIC) questionnaire. The PACIC was developed in 2004 to measure the care experience of patients living with chronic conditions. It allows to measure how much, on a scale from 1 to 5, patient experience with care corresponds to the **Chronic Care Model**. This model was developed by the MacColl Centre for Health Care Innovation and identifies six elements that are essential for a health care system to encourage high-quality chronic disease care:

- Creating an organisation that provides safe and high quality care.
- Mobilising community resources to meet patients' needs.
- Empowering and preparing patients to manage their health.
- Assuring effective, efficient care and self-management support.
- Promoting care that is consistent with scientific data and patient preferences.
- Organising data to facilitate efficient and effective care.

Since its creation, the PACIC has been translated in 11 languages and has been used in more than 130 peer-reviewed studies worldwide, **setting international standards of care quality for chronic conditions**.

In the H-CARE Pilot Survey, the 11 items of the PACIC-S have been adapted for patients and carers (family members...) living with rare or complex diseases, and for specialised care. These adapted versions are called:

- **The PAC-RD, or Patient Assessment of Care for Rare Diseases:** items 1 to 11, except for 9C (Table 1).
- **The CAC-RD, or Carer Assessment of Care for Rare Diseases:** items 1 to 11, including 9C (Table 1).

The H-CARE Pilot Survey also includes questions on:

- Emotional support: items 12 and 12C (Table 1);
- General satisfaction with care: items 13 to 15 (Table 1);
- Experience with diagnostic, with two questions for respondents whose rare diseases has been diagnosed;
- Sociodemographic characteristics, hospital, unit, disease(s) and how respondents heard about the survey.

**70% of ERkNet, eUROGEN, LUNG and Genturis respondents** think that the questions asked in the H-CARE Pilot Survey allowed them to express their satisfaction with the care delivered by their healthcare provider **quite well or very well**.

The PAC-RD has been **partially validated for rare diseases in German, French, English and Spanish**, which means that for those languages:

- It *fits the hypothesised model* for chronic care well (French, English, Spanish) or very well (German) (Table 2).
- Items composing the PAC-RD are related to one another (*internal consistency* - Table 2).
- The mean score for the PAC-RD allows to measure differences between healthcare providers that are part of an ERN and healthcare providers that are not (Table 3), but correlations with other measures of care experience or with clinical assays have not been tested (*construct validity*).
- All aspects of care experience for rare diseases may not be fully covered (*content validity*): if a questionnaire was developed and validated especially for rare diseases, it may include dimensions that are more specific to rare diseases such as emotional support, care coordination, diagnosis, genetic testing or the place of patient organisations in care.
- The PAC-RD cannot be easily adapted to the diversity of rare diseases because respondents should answer to every question, even when they are not adapted to each individual case (for instance respondents should answer questions on treatments even when there is no treatment for their rare disease).

**Table 1. Mean score for items and scales of the H-CARE Pilot Survey ( respondents who assessed healthcare providers that are part of ERNs ErkNet, eUROGEN, LUNG or Genturis N=605)**

	Item number	Items of the H-CARE Pilot Survey	N	Mean (sd) 1-5**
Questions (items) of the PAC-RD and CAC-RD	1	[I was] Given choices about treatments to think about	546	3.5 (1.4)
	2	[I was] Satisfied that my / the patient's care was well organized	592	4.4 (0.9)
	3	[I was] Helped to set specific goals to improve my / the patient's eating or exercise	551	3.5 (1.4)
	4	[I was] Given a copy of my / the patient's treatment plan*	548	3.7 (1.6)
	5	[I was] Encouraged to go to a specific group or class to help me cope with my / the patient's rare or complex disease	543	2.8 (1.6)
	6	[I was] Asked questions, either directly or on a survey, about my / the patient's health habits	569	3.6 (1.5)
	7	[I was] Helped to make a treatment plan* that I / the patient could do in my / his / her daily life	530	3.5 (1.5)
	8	[I was] Helped to plan ahead so I could take care of my / the patient's rare or complex disease even in hard time	532	3.5 (1.5)
	9	[I was] Asked how my / the patient's rare or complex disease affects my / the patient's life	560	3.5 (1.5)
	9C	[I was] Asked how the patient's rare or complex disease affects my [the carer's] life	206	3.1 (1.6)
	10	[I was] Contacted after a visit to see how things were going	548	2.8 (1.6)
11	[I was] Told how my visits with other specialists, like a geneticist or cardiologist, helped my / the patient's treatment	522	3.3 (1.6)	
Overall care experience	PAC-RD	Patient Assessment of Care for Rare Diseases (11 items)	280	3.2 (1.1)
	CAC-RD	Carer Assessment of Care for Rare Diseases (12 items)	162	3.8 (1.0)
Other items of the H-CARE Pilot Survey	12	[Healthcare professionals] Helped me / the patient deal with emotions related to my / his / her health status	515	3.2 (1.5)
	12C	[Healthcare professionals] Helped me [the carer] deal with emotions related to the patient's health status	199	3.2 (1.6)
	13	[I was satisfied with] The outcomes of my / the patient's care and/or treatments	567	4.3 (1.0)
	14	[I was satisfied with] The information I had on the benefits and risks of my / the patient's care and/or treatments	560	4.1 (1.1)
	15	[I was satisfied with] All in all, the care I / the patient received in this unit	571	4.3 (1.0)

\* A treatment plan is a list, made with your care team, of what needs to be done to take care of your health.  
 \*\* 1=None of the Time; 2=A Little of the Time; 3=Some of the Time; 4=Most of the Time; 5=Always  
 N = number of respondents; Mean = average score on a scale from 1 to 5; sd = standard deviation.  
 Questions 9C and 12C were only asked to carers.

**Table 2. Model fit and internal consistency of the PAC-RD in four languages (all respondents N=3699)**

	Number of respondents	Internal consistency Cronbach's $\alpha$	Model fit <i>Confirmatory factor analysis based on polychoric correlation matrix</i>					Sum up model fit
			$\chi^2$ for independent model (df=55)	SB $\chi^2$ (df=44)	RMSEA	CFI	AGFI	
<i>Value good if</i>	<i>&gt; 300</i> <i>&gt; 200</i>	<i><math>\alpha &gt; 0.9</math></i>	<i><math>p &lt; 0.001</math></i> <i><math>p &lt; 0.005</math></i>	<i><math>p &lt; 0.001</math></i> <i><math>p &lt; 0.005</math></i>	<i>&lt; 0.05</i> <i>&lt; 0.08</i>	<i>&gt; 0.99</i> <i>&gt; 0.95</i>	<i>&gt; 0.99</i> <i>&gt; 0.95</i>	
German	359	0.900	7459.66 $p < 0.001$	70.550 $p = 0.0068$	0.041	0.996	1.000	Very good
French	313	0.918	9782.422 $p < 0.001$	89.785 $p < 0.001$	0.058	0.995	1.000	Good
English	309	0.937	14969.99 $p < 0.001$	127.121 $p < 0.001$	0.078	0.994	1.000	Good
Spanish	201	0.941	11446.697 $p < 0.001$	81.017 $p = 0.0006$	0.065	0.997	1.000	Good

Colour code for values: Excellent; Very good; Good; Moderately good

## 2 KEY RESULTS OF THE H-CARE PILOT SURVEY

Patients and carers living with a rare disease affecting kidneys, lungs, the urogenital area and genetic tumour risk syndromes have a better healthcare experience when they are treated by hospital units that are part of a European Reference Network

Table 3. Mean score for the PAC-RD and CAC-RD, on a scale from 1 to 5

### 3.4 Average care experience of patients and carers with healthcare providers that are part of ERNs Erknet, eUROGEN, LUNG or Genturis.

*In the scientific literature on chronic conditions, the highest scores for the PACIC questionnaire are 3.3, which are mostly reached when care teams are multidisciplinary or trained for chronic care and when patients are part of a disease-management program<sup>1</sup>.*

### 2.6 Average care experience of patients and carers with healthcare providers treating rare diseases affecting kidneys, lungs, the urogenital area and genetic tumour risk syndromes and that are not part of an ERN.

*This score is significantly lower than the average score for healthcare providers that are part of an ERN.*

Compared to respondents who evaluated healthcare providers that are not part of an ERN, respondents who assessed ErkNet, eUROGEN, LUNG and Genturis healthcare providers:

- Have been waiting for a shorter amount of time to get a confirmed diagnosis of a rare or a complex disease after they first sought medical advice;
- Have a significantly better experience with their care;
- Are significantly more satisfied with their care.

This is true for all questions of the H-CARE Pilot Survey.

In addition, within respondents who assessed healthcare providers that are part of ErkNet, eUROGEN, LUNG and Genturis mean scores to the PAC-RD and CAC-RD (Table 5):

- Are significantly higher when Spanish, Latvian and Italian HCPs were assessed, when respondents are carers, when they heard about the survey through their hospital and when they are under 18 years old.
- Do not significantly differ depending on gender.

### On-site dissemination of the survey was key for ERNs ErkNet, eUROGEN and Genturis

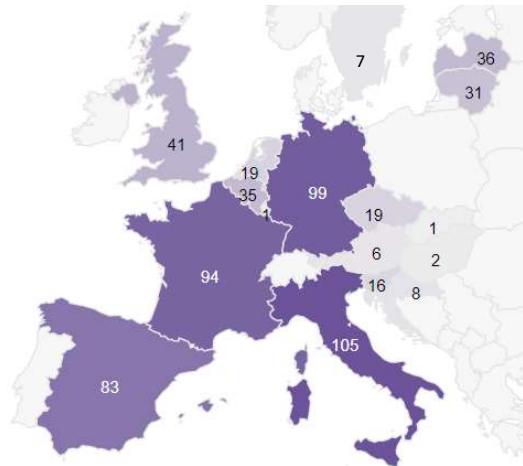
The H-CARE Pilot Survey has been disseminated from December 16<sup>th</sup> 2019 to March 29<sup>th</sup> 2020, online through the Rare Barometer and EURORDIS channels but also on-site, in hospitals, by participating healthcare providers who distributed leaflets and posters to patients and carers. More than half ErkNet, eUROGEN and Genturis respondents were contacted on-site, which shows the importance of an active participation of healthcare providers in recruiting respondents for disease groups that are less reached through online channels.

In addition, it appeared that distributing leaflets and posters is not efficient, hence on-site response rate

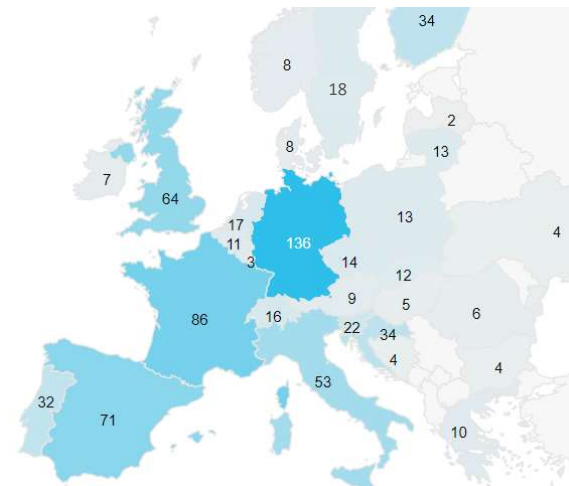
was low. That is why the H-CARE Pilot Survey Working Group proposes:

- To evaluate the use of tablets in HCPs (cost, response rate, organisation...);
- To test the possibility to distribute paper questionnaires to patients and carers, as well as prepaid envelopes that respondents can send directly to a third-party (EURORDIS or other), which will then enter answers into the survey software. This would be more direct ways for respondents to answer the survey while ensuring that their individual answers are not shared with their care team.

<sup>1</sup> Frei et al. 2014, doi: 10.2337/dc13-1429; Ralston et al. 2014, doi: 10.4338/ACI-2013-10-RA-0079; Wolff et al. 2010, doi: 10.1093/geront/gnp124; Stuber et al. 2018, doi: 10.1016/j.ctim.2018.05.006; Tsiachristas et al. 2014, doi: 10.1186/1478-7547-12-17; Chmiel et al. 2017, doi: 10.4414/smw.2017.14522; Szecsenyi et al. 2008, doi: 10.2337/dc07-2104; Gensichen et al. 2011, doi: 10.1007/s10597-010-9340-2.



**Map 1. Number of respondents who assessed HCPs that are part of ERNs ErkNet, eUROGEN, Genturis and LUNG (country of the hospital assessed)**



**Map 2. Number of respondents who assessed HCPs treating rare diseases affecting kidneys, the urogenital area, lung and genetic tumour risk syndromes and that are not part of an ERN (country of the hospital assessed)**

**Table 4. Sample characteristics**

Healthcare providers (HCPs) assessed in the H-CARE Pilot Survey that are part of ERNs ErkNet, eUROGEN, Genturis and LUNG	Healthcare providers (HCPs) assessed in the H-CARE Pilot Survey that are treating rare diseases affecting kidneys, the urogenital area, lungs and genetic tumour risk syndromes and that not part of an ERN
605 respondents.	714 respondents.
4 in 10 respondents were contacted through their care team.	10 in 10 respondents contacted online (50 % patient organisations; 30% EURORDIS channels).
36 participating HCPs, that received individualised results.	HCPs that are not part of ERNs ErkNet, eUROGEN, LUNG or Genturis did not disseminated the survey on-site.
6 in 10 respondents are patients.	7 in 10 respondents are patients.
4 in 10 respondents are 50 years old or over.	4 in 10 respondents are 50 years old or over.
2 in 3 respondents are women.	2 in 3 respondents are women.
2.8 years: mean time to get a diagnosis of a rare or complex disease after they first sought medical advice.	4.1 years: mean time to get a diagnosis of a rare or complex disease after they first sought medical advice.

**Table 5. Relationship between the PAC-RD or the CAC-RD average score and characteristics of respondents who assessed healthcare providers that are part of ErkNet, eUROGEN, Genturis and LUNG**

Characteristics	Relationship	Tendency
Country of the hospital assessed	***	Higher scores when Spanish, Latvian and Italian HCPs were assessed.
Status Patient or carer (family member or other)	***	Higher scores when HCPs were assessed by carers than when they were assessed by patients.
Age	***	Higher scores when respondents are under 18 years old.
How respondents heard about the survey	***	Higher when respondents heard about the survey through their care team
Gender	ns	/

*n.s.* not significant; \* weakly significant ( $p < 0.015$ ); \*\* significant ( $p < 0.005$ ); \*\*\* very significant ( $p < 0.001$ ).

### 3 DEVELOPING A COMMON FEEDBACK MECHANISM

The H-CARE Pilot Survey allowed to better understand how to **set standards of care quality for rare diseases** and **how to develop a Common Feedback Mechanism** to measure patients' and carers' experience with care across the 8000+ rare and complex diseases, across the 24 ERNs and across Member States.

Based on key results presented above, the H-CARE working group will propose a project that would allow to:

- **Develop a model and validate a scale** that would contribute to **set standards of care quality for all rare diseases**, that would include dimensions that are specific to the different types of rare diseases and that would be **flexible enough** to adapt to the diversity of situations and profiles of people living with a rare disease;
- **Ensure robust, comparable and independent data and results by using the same survey methodology** across the 8000+ rare diseases, across 24 ERNs and across Member States;
- Achieve **sufficient survey sample size and coverage** of the ERNs patient population;
- **Avoid duplication of efforts** through pooling of resources (human, financial, tools such as software, etc.) which would result in economies of scale;
- Provide information in order to align strategic decisions and operational delivery of the ERNs with patients' needs and experiences, specifically the development of **healthcare pathways and treatment protocols**.

## THANK YOU

to all people living with a rare disease who participated in the survey, to the healthcare providers and patient representatives of the European Reference Networks ErkNet, eUROGEN, Genturis and LUNG who participated in the design and the dissemination of the survey, and to Rare Barometer partners!