

Centres of expertise for rare diseases: designation process in the Netherlands

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Introduction

In anticipation of the European Reference Networks, the Dutch Ministry of Health, Welfare and Sport appointed the NFU (Netherlands Federation of University Medical Centres) as lead in the development and implementation of a procedure for the designation of rare disease centres of expertise. In close collaboration with Orphanet NL and the VSOP (Dutch National Alliance for Rare and Genetic Diseases representing patient organisations), a NFU project team developed a system to evaluate candidate centres of expertise and officially designate those fulfilling a defined set of criteria. The unique aspect of the Dutch designation process is the combination of the medical/scientific evaluation of centres by medical specialists and the concomitant evaluation by patient organisations, adding the patients perspective.

Scope of the candidate centres of expertise

Classification of rare metabolic diseases	
Rare inborn errors of metabolism	
Lysosomal disease	
Sphingolipidosis	
Niemann-Pick disease type E	
Encephalopathy due to prosaposin deficiency	
Gangliosidosis	
GM1 gangliosidosis	
GM1 gangliosidosis type 1	
GM1 gangliosidosis type 2	
GM1 gangliosidosis type 3	

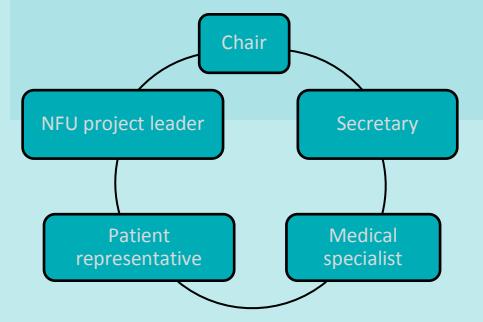
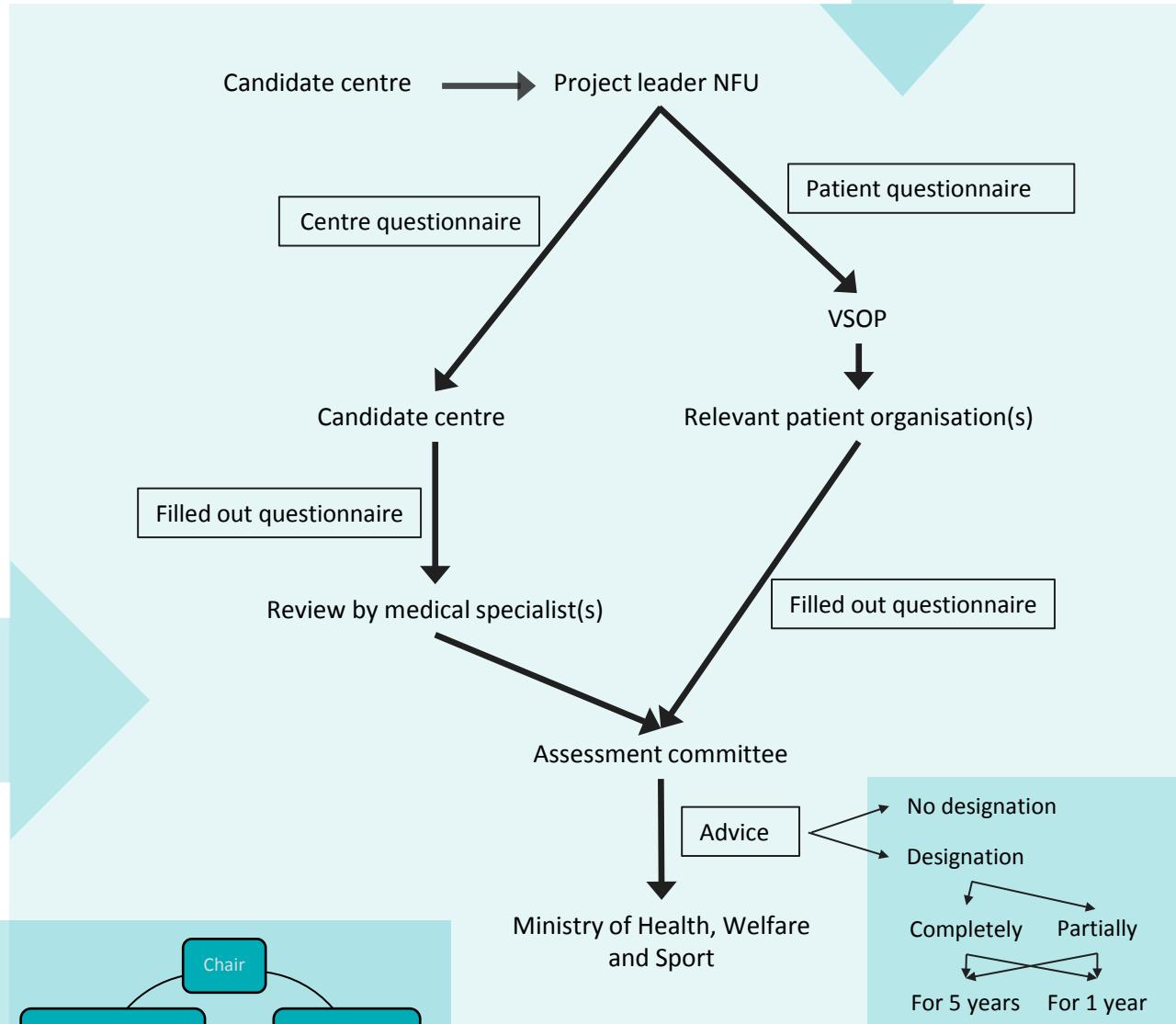
The centres could apply for specific diseases and/or disease groups as listed in the Orphanet classifications

Method

Theme	Criteria
Quality of care	Highly specialized complex patient care
	Care standards and guidelines
	System for safeguarding quality of care
	Multidisciplinary team
	Care provision within the entire chain
	Scientific developments
Transition of care	Transition from childhood into adulthood
Continuity	Transfer of knowledge within the centre
	Accredited by the hospital Board of Directors
	Open to auditing
Cooperation	With patients and patient organisations
	Other (inter)national centers of expertise
Information and communication	Point of information
	Education/ Awareness raising
Research	Scientific research
	Patient registration/Biobank
Cross-border healthcare	Participation in cross-border healthcare

Set of criteria used to evaluate candidate centres of expertise

The set consists of the EUCERD criteria in combination with specific criteria from the Dutch Rare Disease Plan.



Assessment committee

This committee consists of an independent chair, the NFU project leader, a secretary, a patient representative from the VSOP and a medical specialist from the Orphanet NL Scientific Advisory Board.

Results

In 2015, of 417 candidate centres 302 centres were designated, either completely or partially:

- 196 centres were designated for 5 years
- 83 centres were designated for 1 year
- 23 centres were partly designated for 5 years and partly for 1 year

:: 302 Result(s)	
Caption : designated centres of expertise =	
NETHERLANDS Gelderland GROESBEEK	Center for Mycobacterial Diseases Radboudumc, locatie UCCZ Dekkerswald More information
NETHERLANDS Gelderland NIJMEGEN	Malignant Hyperthermia Investigation Unit Nijmegen CWZ - Canisius Wilhelmina Ziekenhuis More information
NETHERLANDS Gelderland NIJMEGEN	Radboud Adrenal Centre Radboudumc - Radboud universitair medisch centrum More information

Conclusion

The unique collaboration of medical specialists and patient organisations in this Dutch designation procedure resulted in a joint decision process and forms the basis for future collaborations between all relevant stakeholders in the Dutch rare disease field.

Publication of centres of expertise

The centres of expertise are published on the Orphanet website www.orpha.net, the Erfocentrum website www.erfelijkheid.nl and the VSOP website www.zichtopzeldzaam.nl.