Scope of vignette:
- authorised products (with marketing authorisation)
- decision process about routine use (and not individual requests for reimbursement)
- submissions for P&R made by manufacturers

Green = related to/any special considerations for rare disease and ultra-rare disease treatments

Portugal	**Standard process (non-orphan drugs)**
Overview of health system and P&R/HTA process	Tax based health system [1] Ministry of health or INFARMED - National Authority of Medicines and Health Products (government agency accountable to the Ministry of Health) monitors, assesses and regulates all activities related to human medicines and health products, makes final reimbursement decision. [2, 5] National Health Technology Assessment System (Sistema Nacional de Avaliação de Tecnologias de Saúde para Portugal - SiNATS) responsible for HTA process. [3, 6] The Committee for Health Technology Assessment (CATS) is an advisory body that is part of the evaluation procedure.
Differentiation of rare disease treatments in the P&R system	None
Eligible medicines	New medicines [6]
Process	Involves: - Submission of application and assessment for quality, safety, efficacy - Evaluation of key considered domains and pricing via peer review, applicant hearings, and input from all assessors - Following evaluation is a negotiation phase and a decision with any necessary terms/conditions - Following negotiation is an agreement and monitoring of 'real-effectiveness' [6]
Disease specific expert input (e.g. clinicians or patients in any stage of the process)	CATS issues recommendations related to the assessment/re-assessment of health technologies, in terms of financing, use or installation by the NHS, in particular therapeutic added value and cost-effectiveness, among other evaluation criteria. The clinical expert or experts that issue these recommendations are chosen according to their expertise about the disease. Infarmed has been developing new tools that will contribute to a closer relationship with the citizens and patients, through the dissemination of information on social networks (e.g. Facebook)

Portugal	Standard process (non-orphan drugs)
	and in 2017, through the creation of the Project INCLUIR (instrument for liaising with patients/citizens or their representative). Patient or patient representative participation is carried out on a case-by-case basis, depending on the specific nature of each consultation process. Priority for patient involvement is in the process of evaluation, and identifying measures of effectiveness and quality of life considered relevant. Infarmed and the National School of Public Health (New University of Lisbon), are carrying out training actions directed at patients and representatives, to construct more informed positions on the processes in which Infarmed intervenes and for Infarmed to include in its processes / activities the perspective of the patient, caregivers and relatives, their experience, needs and preferences.
Key domains in assessment	- Clinical effectiveness [4, 6] - Cost effectiveness [5, 6] - Other (dimensions of the technology value (including affordability - HTA usually assess the pharmacotherapeutic benefit, and the economic advantage of the intervention compared to its alternative(s), including affordability (e.g. taking into consideration the budget impact.))
Evidentiary requirements	For RDTs, more leniency is informally permitted for evidentiary requirements when assessing the clinical and economic advantages, but especially when it's not possible to undertake formal economic studies. Usually, when it's not possible to undertake formal economic studies, companies are asked to submit a simple cost-consequence study, including annual cost of treatment (short term) and patient health gains (survival and quality of life data).
PROMs	Not used
Appraisal framework	There is no pre-defined appraisal criteria. SiNATS makes decisions on - Inclusion/withdrawal from use listings - Maximum price - Cost limitation (Negotiation and managed entry agreements) - Additional monitoring of use SiNATS contributes to the process of - Public tenders [5, 6]
Reimbursement decision	Outpatient setting: General scheme: 4 levels of reimbursement: a) 90%, b) 69%, c) 37%, d) 15%. Based on therapeutic classification Specific scheme: 2 options:

Portugal	Standard process (non-orphan drugs)
	1. Population group specific: extra reimbursement (15%) for pensioners 2. Disease specific: defined pathologies (e.g. Alzheimer) Inpatient setting: Disease specific: fully financed (100%) (e.g. HIV, Crohn disease, Multiple Sclerosis) RDTs do not automatically fall into a specific category of reimbursement.
Pricing process	External reference pricing Internal reference pricing applies only when there is at least one generic on the market, which is not applicable to rare disease treatments. Pricing could be a two-step process: External reference pricing sets the maximum price of medicines (non-generics prescription-only medicines). In case they want to be reimbursed, the MAH must apply for reimbursement. At this time, they have to be compared to the price of the alternatives, if available (2nd step). Additionally, price and reimbursement can be set together.
Managed entry agreements	- Risk sharing agreements (financial and performance based) - Conditions of use - Reassessment of technologies on the market - Conditional reimbursement [5]
Main challenges in appraising medicines for rare diseases (tick all that apply)	X Lack of good quality clinical data X Lack of real world data X Introducing value for money X Monitoring treatment efficacy X Managing budget impact X Lack of criteria/transparency of OMP P&R processes X Making arrangements to work for all stakeholders X Lack of long-term meaningful outcomes X Other, please specify
Impact of special processes	N/A
Proposed policy change	None
Joint initiatives	None
SOURCES	
1	https://healthmanagement.org/c/hospital/issuearticle/the-portuguese-healthcare-system-universal-and-comprehensive-1
2	http://www.infarmed.pt/web/infarmed-en/human-medicines

Portugal	Standard process (non-orphan drugs)
3	http://www.infarmed.pt/documents/15786/1963929/SiNATS/4f9df178-482b-4f37-a15a-02041b4d3c48
4	http://www.apdh.pt/sites/apdh.pt/files/2015IHFChicago_JOAO%20MARTINS%20(2).pdf
5	http://www.infarmed.pt/documents/281/1432055/PCAEC04_vering.pdf
6	http://whocc.goeg.at/Literaturliste/Dokumente/CountryInformationPosters/Portugal2015.pdf

Created in May 2019 by the IMPACT-HTA team with the support of the country experts. Last updated in September 2019.

Acknowledgments: We would like to thank Claudia Santos, Data Analyst from the Information and Strategic Planning Department at INFARMED, for her time and valuable contribution in providing the information used to create and validate this vignette. This research is funded under the EC's Horizon 2020 Programme within IMPACT-HTA. Results reflect the authors' views. The EC is not liable for any use of the information communicated.

This vignette was compiled based on information provided by country experts and desk research. The information provided may be incomplete or contain inaccuracies. If you have any comments or updates, please email us at the following email addresses:

- Elena Nicod at elena.nicod@unibocconi.it
- Amanda Whittal at amanda.whittal@unibocconi.it