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

Lithuania	**Standard process (non-orphan drugs) - Common Reimbursement Process (CDR)**	**Special processes (orphan and ultra-orphan drugs)**
Overview of health system and P&R/HTA process	Tax based system [1] National level: The SMCA (safety and effectiveness committee), the Ministry of Health and the NHIF (payer) are the main actors in the regulation of pharmaceuticals. The final decision is made by the Minister of Health (supported by the technical evaluations from the Pharmaceuticals Reimbursement Commission and the NHIF) regarding strategic planning, and whether a product will be reimbursed and at what price. The Pharmaceuticals Reimbursement Committee (representatives from the Ministry of Health and other ministries - labour, social affair finance) advises the Minister of Health on reimbursement decisions. The SMCA carries out regulatory and control functions by granting marketing authorization, classifying prescription status, conducting pharmacovigilance, inspecting the pharmaceutical industry and pharmaceutical product distribution companies, controlling the quality and advertising of pharmaceuticals and supervising clinical trials, performs the HTA of medicines only. The SMCA also registers pharmaceuticals and keeps a list of licenses of pharmaceutical companies, pharmacies and pharmacists. The NHIF is in charge of contracting pharmacies and reimbursing medicine costs, as well as for procuring high-cost pharmaceuticals via public tenders. [2]	Tax based system [1] In addition to the Standard Process overview, reimbursement for OMPs is regulated by a special order of the Ministry of Health. For OMPs, the Very Rare Conditions Committee (VRCC) is specified as a decision making body in the process.

Lithuania	Standard process (non-orphan drugs) - Common Reimbursement Process (CDR)	Special processes (orphan and ultra-orphan drugs)
	The MoH issued order No. 159, regulates the inclusion process for placing medicines on a reimbursable (positive) list. [3]	
Differentiation of rare disease treatments in the P&R system	EMA orphan designation Ultra-OMP designation: Very rare (not more than one newly diagnosed case in 200 000 of Lithuanian population per year) human health condition (further – very rare condition) is deemed to be a health disorder, which is life-threatening and/or causing significant permanent disability, which may be subject to an effective etiological (a factor affecting the onset of the disease) or a pathogenic (the factor responsible for the clinical course of the disease) treatment, when the treatment costs of this very rare condition are not reimbursed, and when the treatment can increase the patient's survival and (or) reduce disability (or prevent the increase in disability).	
Eligible medicines	The application drives both the special process and CDR process. Products are not otherwise selected for the special (very rare condition) process. If there is no application either to the special or common-reimbursement process, the product is not appraised in either of the processes. Without application and consequent appraisal, the product is not reimbursed and could only be accessed commercially.	
Process	The MoH issued order No. 159 outlines a special application form template for medicines, the guidelines for appraising the application, and a reimbursement decision-making approach. [3] The order sets the process rules for standard process->according to the order the standard process: - Starts with the submission (set of documents about efficacy effectiveness evidence et budget impact et cetera) from industry to MoH - An application is made - The three key domains are assessed - Decision is made according to points - “Permission for NHIF to pay” starts after signing the act (by Minister) which permits inclusion on positive list	The MoH issued an order for the treatment reimbursing “rules” of very rare conditions: The individual patient case-based decision is made by VRCC based on hospital application (hospital acts as applicant) to VRCC for individual patient treating. The decision to include specific medicine for the treatment of very rare condition into special reimbursement list is made by VRCC based on university hospital (or the physicians society) application to VRCC. The main differences of special process (for very rare conditions) appraisal from CDR process are: - Therapeutic value is not graded - No waiting list in case of positive (to reimburse medicine for very rare condition) decision

Lithuania	Standard process (non-orphan drugs) - Common Reimbursement Process (CDR)	Special processes (orphan and ultra-orphan drugs)
Disease specific expert input (e.g. clinicians or patients in any stage of the process)	Specialty medical societies act as providers of the opinion about the positioning of the patient (i.e. sub-group) within the indication under review	University hospital representing physicians (Concilium) act as submitters of individual patient application. For OMPs, the Very Rare Conditions Committee (VRCC) is specified as a decision making body and the process flow.
Key domains in assessment	- Clinical efficacy (Medical benefit - Effectiveness, safety and severity of the disease treated) - Cost-effectiveness (Results of pharmacoeconomic evaluation) - Budget impact [2, 3]	More leniency is formally accepted quality of evidence submitted for OMPs
Evidentiary requirements	"Scientific publications" (no requirement for systemic literature review)– *usually pivotal trials publication.*	"Peer reviewed publications"
PROMs	None	
Appraisal framework	1) An assessor (clinical; based in SMCA) assigns a score from 5–15 to a medicine's therapeutic value (TV); 2) An assessor (economical; based in MoH Pharmacy Dep) assigns a score from 1.5–7.5 to a medicine's pharmacoeconomic value (PEV); 3) An appraisal of the budget impact (BI) calculations provided within application and recalculating the actual BI [3] (by NHIF)	The individual patient case-based decision should include evidence on: 1. The health condition under review meets the definition of very rare condition 2. The medicine under review is meets a set of criteria, which are predefined in the order of MoH (authorization details, efficacy evidence et cetera) 3. The hospital physicians board opinion and evidence (individual medical case history and diagnosis, official data on prevalence, if not existent – orphan code or/and publication) that individual patient's current health state under review is actually a very rare condition under review.
Reimbursement decision	The positive decision-making approach is based on counting various combinations of the TV scores, PEV scores, and BI conclusions.	The decision to include specific medicine for the treatment of very rare condition into special reimbursement list should include evidence on:

Lithuania	Standard process (non-orphan drugs) - Common Reimbursement Process (CDR)	Special processes (orphan and ultra-orphan drugs)
	There are three possibilities: 1) the TV is 9 points, PEV is at least 4 points, BI is at least 5 percent less than that (medicine costs only) of comparator; 2) TV is 10 points, PEV is at least 4 points, and BI will not increase expenditure for the NHIF; 3) TV is at least 11 points, PEV is at least 4 points, and BI is equal to a maximum of 0.05% of the previous year's NHIF expenditure on all reimbursed medicines (if BI exceeds the 0.05% criteria, the medicine is included on a "waiting list"). [3]	1. The health condition under review meets the definition of very rare condition 2. The medicine under review meets a set of criteria, which are predefined in the order of MoH (authorization details, efficacy evidence et cetera) 3. Treatment rules for the very rare condition under review Two decision options depending on which of the two above processes was initiated: 1. decision to reimburse the treatment of very rare condition with specific medicine to the individual patient (individual case-based). 2. decision to reimburse the treatment of very rare condition with particular medicine to all patients, i.e. including medicine into special positive list, specifying the treatment positioning (i.e. indication, diagnosing requirements) and treatment rules (eligibility, treatment lines and starting-stopping rules). The decision for each process is either positive or negative.
Pricing process	Rules apply for standard process pricing Reference pricing (Pharmaceuticals are grouped on the basis of the International Non-proprietary Name (INN), method of use, form, purpose and length of action. --> The reference price for the group is the cheapest priced product in the group). Since 2008 there have been price volume agreements for new pharmaceuticals. [2]	Different rules apply for rare disease process pricing: In the common process, it is based on international reference pricing and some rules (i.e. not more than 95% on the average of the three cheapest) In the RDT individual process: there are some treatment pricing rules (i.e. the limit that can be spent for each individual is determined based on the disease). Each calendar year has a special budget for this particular process.

Lithuania	Standard process (non-orphan drugs) - Common Reimbursement Process (CDR)	Special processes (orphan and ultra-orphan drugs)
Managed entry agreements	- Confidential discount - Outcome based scheme for individual patients, only paying for certain performance	- Confidential discount - Budget cap - Outcome based scheme for individual patients, only paying for certain performance
Main challenges in appraising medicines for rare diseases	- Lack of real world data - Introducing value for money - Monitoring treatment efficacy - Managing budget impact - Lack of criteria/transparency of OMP P&R processes - Making arrangements to work for all stakeholders - Lack of long-term meaningful outcomes	
Impact of special processes	- Currently, 9 medicines are included in special (very rare condition treatment) reimbursement list - Altogether 136 applications for individual patient treatment were submitted in 2016, oncology treatment was the subject in 44% of them.	
Proposed policy change	New common-reimbursement process (preliminary start May 2019) will take into account QALY ICER and will employ PAS. This will probably change the attitude toward the decision basis in special process for very rare conditions.	
Joint initiatives	SMCA has been granted full membership per acclamation to the Consortium that implements the EUnetHTA JA3 project at 12 10 2016 (*comment – there was no activity from SMCA in this project*).	
SOURCES		
1	http://www.vlk.lt/sites/en/health-insurance-in-Lithuania/health-insurance-system	
2	http://www.who.int/health-laws/countries/ltu-en.pdf	
3	An overview of HTA legislation in Lithuania.doc	

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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:

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