A National Approach to Reimbursement Decision-Making on Drugs for Rare Diseases in Canada?
Insights from Across the Ponds

Démarche nationale quant aux décisions de remboursement des médicaments pour maladies rares au Canada?
Pistes provenant d'outremer

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Table B4. Public accountability and decision implementation considerations

Country	Transparency	Appeals mechanisms	Reassessment or review of decisions	Conditions of implementation	Funding Mechanism	Role of stakeholders
Australia *General Reimbursement-The Pharmaceutical Benefits Scheme* (PBAC 2008b, 2013b)	PBAC is conscious of the need to be as open as possible in its proceedings, consistent with the secrecy provisions of the Act. It therefore provides to sponsors all relevant documents and evaluations considered by the committee. It also allows up to two sets of written pre-PBAC consultation documents from each sponsor in relation to its submission for a product, as well as a hearing before the committee when it is considering advice from its subcommittees. Verbal advice of the PBAC decision on application on first Wednesday after the PBAC meeting and written advice 15 working days after the PBAC meetings PBAC outcomes published on Department website 6 weeks after the PBAC meeting	• Applicant may formally request independent review of decision Opportunity to discuss unsuccessful submission with Chair of the PBAC available to canvass opportunities	If PBAC does not make a recommendation to list, the applicant may re-submit with new or additional information	Drugs that receive a negative recommendation from PBAC cannot be funded.	RSAs may be proposed by the sponsor to PBAC, the Pharmaceutical Benefits Pricing Authority (PBPA) or DoH. They may be recommended by PBAC (usually in relation to cost-	Appeal recommendations: • Applicant Re-submit application: Applicant Provide comments on public summary document before posted on website:

	Public summary documents published to the Department website—opportunity for sponsor to comment on the PSD before it is published (3) Formal letter advising that the listing is approved one more prior to the listing date	for resubmission and/or independent review (no temporal incentive or disincentive for sponsor to seek a review in preference to making resubmission) Independent reviewer (not affiliated with PBAC or involved in initial review) appointed by the convener (convener does not contribute to the content or findings of a review) • Independent reviewer (not affiliated with PBAC or involved in initial review) appointed by a convenor (convenor does not contribute to the content			effectiveness and/or health outcomes) or by PBPA or the department (usually in relation to overall costs). Given that RSAs typically rely on utilization data, there are advantages to all parties if a sponsor puts any proposal for such an arrangement early in the process of application for a drug listing (e.g., in its submission to PBAC), rather than introducing the proposal later. RSAs (also previously called "price-volume agreements") have been developed to address at least	• Applicant

		or findings of a review) Possible to contract a Secondary Reviewer to work in conjunction with the Reviewer if there are disparate issues in contention or if issues outside of the area of expertise			three types of risk	
Australia *Life Saving Drugs Program (LSDP)* (AGDH 2011; PBAC 2008d; AGDH 2013c)	Sponsor required to submit draft Condition Guidelines when commencing the LSDP submission process. PBAC may comment on the Condition Guidelines, and should the drug be approved for funding, the draft Condition Guidelines will be reviewed in consultation with the DAC and finalised by the Department. http://www.health.gov.au/internet/main/publishing.nsf/Content/lsdp-administrative-requirements	If patient, their parent/guardian, or treating physician is unhappy with decision made under the LSDP, it may be discussed with the LSDP Secretariat. This is an opportunity to correct any misunderstandings and present any new information or evidence If unresolved, a	Patient eligibility will be reviewed in accordance with the frequency set out in the relevant drug/condition LSDP Guidelines, but generally 12 months after commencing therapy and every 12 months thereafter. Continued eligibility subject to the assessment of	Timeframes for a Government decision dependent on business of the day and Government priorities at the time Australia generally supports use of Disease Registries which allow the Advisory Committee to monitor patient progress to treatment	2012 Review of the program recommended risk sharing arrangements between the Government and drug suppliers to provide the Government with greater certainty on the implications of funding a medicine through the LSDP	Submit Condition Guidelines: Sponsor Comment and review on Condition Guidelines: PBAC DAC Department Initiate appeal: Patient/Guardian Treating physician

		review of the decision may be requested within 60 days of the original decision. The First Assistant Secretary, Pharmaceutical Benefits Division, is responsible for reviewing decisions.	evidence, which demonstrates: clinical improvement in patient or stabilization of the patient's condition. DAC is responsible for determining patient continuation of therapy through the LSDP			
Australia *Highly Specialised Drugs Program (HSDP) (Mabbott et al. 2010)*	See PBAC General	See PBAC General	See PBAC General	See PBAC General	See PBAC General	See PBAC General
Austria *General reimbursement: Extramural drugs* (Austrian Federation of Social Insurance Institutions 2007; Bucholz 2009; PHARMIG 2013)	The EKO is updated monthly via the Internet (www.avsv.at) and is published in hard copy twice a year Manufacturer's submission and HEK evaluation of submission not made publicly available	In the case of a negative decision or change in a positive decision, delisting, or new restrictions, the manufacturer may appeal to the Independent Drug Commission	No formal process for regular review of decisions If new pharmacological, medical-therapeutic or economic evidence is found, an application for the delisting or changes in the listing of a	All newly submitted pharmaceuticals are temporarily listed ("red box") for 24-36 months during HEK evaluation and price setting; during this time period, reimbursement can be requested on case-by-case basis by the prescribing physician Once decision to fund has been made, pharmaceuticals listed on a positive list (green	Insurance-based	Appeal recommendations: • Applicant Independent Drug Commission *(oversees HVB and HEK)* can veto decisions

			pharmaceutical can be proposed by the manufacture; decisions to delist or change the listing of a pharmaceutical can be made by HVB	if no conditions, yellow if conditions) Timeline for review and reimbursement decision: 180 days		
Austria *General reimbursement: Intramural drugs* (Austrian Federation of Social Insurance Institutions 2007)	Drug commissions respond back to the clinician who requested the drug (informal process)	No information found	No information found	No information found	Hospital budget	No information found
Austria *Individual (case-by-case) reimbursement*	No information found	No information found	No information found	No information found	Insurance-based	No information found
Belgium *General Reimbursement* (Denis et al. 2009; Bogaert and Klasa 2009; Denis et al. 2011)	Drug reimbursement dossiers are not publicly available, but are confidential reports retained by the DRC	No formal appeal mechanism	• The reimbursement decision can be subject to a reappraisal procedure which will revise or confirm the pharmaceutical product reimbursement	The reimbursement conditions: conditions limiting the access to reimbursement, e.g., age range, preliminary diagnostic examinations, maximum dosage, etc.	Orphan drugs are fully reimbursed through the NIHDI: • A compulsory health insurance system, which is funded from health insurance	No information found

			modalities. Reappraisals are either individual or collective and are usually triggered by budget impact or uncertainty concerns. Individual reappraisal usually takes place between 18 and 36 months after the positive decision. • Companies need to submit a revised dossier to the DRC after 1.5 to 3 years following initial reimbursement approval		contributions and general taxation, out-of-pocket costs, and from other external sources, such as a contribution of premiums paid for complementary health insurance, a yearly license fee chargeable to pharmaceutical companies and a levy on the turnover of pharmaceutical companies on the Belgian market.	
Belgium *Special*	The decisions from CMDOD on the cases delegated to the local sickness funds are not systematically reported to the SSF. Hence there is no control on the uniformity	If the patient disagrees with the decisions	Individuals need to renew applications for	Request for reimbursement must be done within three years	(SSF) Compulsory health system	Appeal recommendations:

Solidarity Fund (SSF)	of those decisions. Another issue hampering transparency is that there is no legal obligation to make the SSF annual report publicly available.	of the SSF, he can launch an appeal to the labour court. Labour courts solely have the competence to judge the reasonability of decision-making, not the content of the decision. This implies that a judge can annul the decision of the College on the allocated amount but not judge on the amount itself.	continuity of SSF funding One member of the CMDOD is delegated the responsibility of decision-making for renewal applications. In some cases, the local sickness fund is delegated the decision-making	following the end of treatment	reimburses under the provision of the NIHDI As the SSF has a closed budget, it may limit the reimbursement of the costs to a percentage of the total cost. Mostly a percentage of 60 % or 75% is used. If the SSF reimbursed up to 75% the total personal share of the patient is limited to an amount varying between €1000 and €1500 annually.	• The applicant
Denmark *General Reimbursement* (Guillaume et al. 2010; Denis et al. 2009)	The reimbursement committee publishes the following on the health authority website: • Members names, and their declaration of interests • Recommendations regarding general reimbursement application from the companies • Their annual report • Their meetings minutes • Reassessment reports	Upon decision-making by the reimbursement advisory committee, if the decision doesn't appeal to one of the members, the member can	Systematic reassessment process is in place (adopted by the Danish parliament) for all the medicines that were granted general	No information found	National public health insurance (a universal and tax funded system financing drugs)	No information found

		demand that this be recorded in the meeting minutes (No further information found)	reimbursement status. Prioritization criteria for the medicines to be reassessed are: • significance of the medicine to the primary sector • public health aspects • new evidence based recommendations • high costs for patients and regions, as well as high consumptions			
Denmark *Individual Reimbursement* (Moller 2003)	No information found	No information found	No information found	• For some types of medicines, the grant may be limited in time • The requesting physician, in case of individual reimbursement is obliged to report back to the Danish medicines agency and adverse effects of the medicinal	National public health insurance	No information found

				product.		
Finland *General Reimbursement* (Ayme and Rodwell 2013c; Mossialos et al. 2013)	PPB shares its draft decision with the applicant. Further evidence may be submitted at this time.	**General Process** Appeal process sends claims directly to the Supreme Court, which considers whether due process has been followed but does not pass judgment based on evidence. **Individual Applications** Can appeal to Kela if the decision received on this application is thought to be wrong. Appeal to Kela within 30 days of the date the written decision is received, and it will be re-examined by Kela. If Kela declines to	In principle, drug reviews take place after three, but up to a maximum of five, years. After a drug review, the PPB can choose to terminate the wholesale price and reimbursement status of the drug. Before this submission, the PPB must hear submissions from the holder of the market authorization and Kela and they must assess the therapeutic value of the drug and the reasonability of the wholesale price on the basis of the new information.	Reimbursement and pricing decisions take up to 180 days A reimbursable new drug that meets the necessary criteria is placed in the basic refund category. It moves to one of the special refund categories when the company has submitted evidence on its therapeutic value and cost effectiveness. In practice, drugs are sold in the basic category for an average of two years. This has been seen as limiting drug choice for the treatment of the most serious illnesses. In very few cases a drug has been granted a higher level of reimbursement immediately.	Basic category of drug: 35% of purchasing price Special higher refund category: 100% of purchasing price Special lower refund category: 65% of purchasing price	Submit further evidence for consideration: • Applicant Initiate appeal: • Applicant Submit information before terminating reimbursement status: • Kela • Manufacturer

		amend the decision, it will forward the appeal to the Social Security Appeal Board. Individuals have a further right of appeal to the Insurance Court (highest court of appeal).				
France *General Reimbursement* (Chicoye et al. 2000; Pelen 2000)	Evaluation report, final recommendation and decision, and results of voting and key issues at expert committee meetings are published on HAS' website	Manufacturer may appeal recommendation and final decision Once final decision made, manufacturer may appeal through public court	Yes, automatic reassessment every 3 years, initially, and every 5 years thereafter	Once decision to fund has been made, pharmaceuticals listed on a positive list with different reimbursement levels Timeline for review and reimbursement decision: 90 (inpatient) to 180 (outpatient) days; for fast-track assessments, reimbursement decision made within 15 days after market authorization	Insurance-based	Appeal recommendations or final decision: • Manufacturer
France *Individual (case-by-case) or cohort reimbursement* (Natz and Campion 2012)	**1) Temporary use authorization (ATU) (individual or cohort)** No information found	No information found	No formal reassessment However, once authorized, pharmaceutical must be reviewed through standard reimbursement	Funding is provided for up to one year (cohort reimbursement) or for the treatment duration (individual reimbursement); periodic reporting of safety and efficacy is required; pharmaceutical	Insurance-based (payment by manufacturer in some cases)	No information found

			process	can be provided according to specified protocol and through hospital pharmacies only		
France *Individual (case-by-case) or cohort reimbursement* (Natz and Campion 2012)	**2) Recommendation for therapeutic use (RTU) (cohort)** No information found	No information found	No information found	No information found	Insurance-based	No information found
France *Individual (case-by-case) or cohort reimbursement* (Natz and Campion 2012)	**3) Temporary protocol of treatment (PTT)** No information found	No information found	No information found	No information found	Insurance-based	No information found
Germany *General Reimbursement* (Fulda 2011; Holtorf et al. 2009).	The G-BA publishes all decisions in detail on its website (www.g-ba.de); pharmaceutical benefit assessments and decisions are also available in English. The German Institute of Medical Documentation and Information ensures maintenance of a publicly accessible version of the assessment report, the summary of product characteristics and the package leaflet.	Appeals to G-BA's decisions are possible on evidence as well as on legal grounds, however EMA orphan drugs are approved for reimbursement (appeals unnecessary)	Once an orphan drug generates annual gross sales of €50 million or more at the cost of the German public health system, the G-BA may request current data on the additional benefit, which the originator has to provide within three months. In which case, the	No information found	Statutory health insurance (GKV)	No information found

			procedure for reimbursement is the same for all other pharmaceuticals .			
Germany *Compassionate Use* (Federal Ministry of Health 2010)	No information found	No information found	• The compassionate use program shall end with the early termination by the responsible person or with the normal availability of the medicinal product on the market, however, at the latest one year after the receipt of the confirmed notification or the authorization required • A re-notification is permissible. In the	The responsible person is obliged to inform the competent federal authority of: • every suspected case of a serious side-effects • the early termination of the compassionate use program • a safety report after completion of the compassionate use program containing and evaluating, in particular, all of the serious side-effects and all of the non-serious unexpected side-effects • new expert opinions and findings from other compassionate use programs which are being conducted in another Member State of the European Union or in another State Party to the Agreement of the European Economic	Reimbursed by the Statutory health insurance (GKV) on the following conditions: • the drug will be used to treat a life-threatening or fatal disease • there is an absence of pharmaceutical therapy with a marketing authorization in Germany • there is scientific evidence of positive therapeutic effects By law	No information found

			process, reference may be made to documents already submitted, in so far as no changes have taken place with respect to them.	Area • changes relating to the therapeutic indication, the strength or the pharmaceutical form of the medicinal product within the framework of the compassionate use program, as well as changes which are capable of having an effect on the safety of the patients	dispensing of the medicinal products in "compassionate use" programs must be free of charge and exempts these products from the prescription medicine pharmacy chain of distribution	
Iceland *General Reimbursement* Icelandic (Medicine Pricing and Reimbursement Committee 2013)	The Icelandic drug price catalogue is published every month and can be found on the committee website: http://www.lgn.is/?pageid=10 Pharmaceutical suppliers must request for publication of information in the Drug Catalogue and the Price List. Application for request of publication must be submitted to the Icelandic Medicines Agency and the Icelandic Medicine Pricing and Reimbursement Committee. A medicinal product may only be marketed once it has been published in the Drug Catalogue and a valid Price List	The committee's decisions cannot be altered, even by the Ministry of Welfare and any disputes have to be taken up in the court of law	Wholesalers' prices are reviewed and adjusted at least every 2 years	Timeframe for reimbursement decision is: 90 days. Timeframe for joint application for reimbursement and Price are 180 days.	• Public health insurance (Tryggingastofnun rikisins, TR), which covers the entire population of Iceland • Financed by general taxation • Patient co-payments for certain categories of drugs	Appeal recommendations: • Applicant
Ireland *General Reimbursement-*	Final appraisals posted to NCPE website.	Any person required to be notified of decisions may	Where reimbursement of a new medicine is	New medicines, including new presentations and applications, granted a	As a result of the 2012 Industry-Department of	Appeal recommendations: • Applicant

Community Drug Schemes (Arthur Cox 2012)		seek a review of the proposal by an expert committee, which review will be carried out within the time frame sat in the legislation for making representations to the Executive. Membership of the expert committee will be agreed between IPHA, the HSE and the DoH. The committee's decision will be made within 60 days and will be accepted as binding.	refused on appeal, and where significant new evidence becomes available subsequently, the applicant can seek a new Pharmacoeconomic assessment.	marketing authorization by the Irish Medicines Board or European Commission will become reimbursable in the Schemes, within 75 days of the date of the reimbursement application. Products which are subject to Pharmacoeconomic assessment will become reimbursable in the schemes within 45 days of a positive HTA decision.	Health recent supply terms and pricing agreement, a budget for innovative and new treatments was created and it is the view that emerging high cost drugs will fall into this budget.	Seek review of decision: • Manufacturers • Others who are required to be notified of the decision
Ireland *Named Patient Regime* (Kela 2013)	No information found	No information found	No information found	No information found	Funding available through HSE (8)	No information found
Italy *General Reimbursement* (Folino-Gallo et al. 2008;	Decision and rationale published on AIFA website (updated every 6 months) and in the Official Journal of the Italian Republic	No information found	No formal process for regular review of fund or do not fund decisions	Once decision to fund has been made, pharmaceutical is listed on a positive reimbursement list	Insurance-based	No information found

Bakowska et al. 2011)			Scheduled reassessment of pharmaceuticals funded with conditions or additional data collection	Timeline for review and reimbursement decision: 180 days		
Italy *Individual or cohort reimbursement (Law 648/96)* (Barham 2012)	List of drugs approved under this law with their indication are published on AIFA website; list is periodically updated	No information found	No information found	Once decision to fund has been made, pharmaceutical is included on a positive list and is prescribed subject to continued surveillance	Insurance-based	No information found
Italy *Individual reimbursement (Law Decree 23/98)* (Barham 2012)	No information found	No information found	No information found	Funding is non-continuous; no further information found	Insurance-based	No information found
Italy *Individual (case-by-case) reimbursement (AIFA 5% Fund)* (Barham 2012)	No information found	No information found	No information found	Funding is provided for up to 6 months; no further information found	Regional Health Authorities or Specialized Program (AIFA 5% Fund) • administered by AIFA; fund comprising mandatory pharmaceutical company donations	No information found
Japan (Liu et al. 2009; Nagae 2012; Orphanet 2009)	No information found	Appeals can be made to the Drug Pricing Organization	Orphan drugs are re-examined after 10 years of approval (all other pharmaceuticals	Reimbursement decisions are made no later than 90 days after approval	National Health Insurance Drug Price List	Appeal recommendations: • Applicant

			reviewed every 2 years)			
Korea (Ngorsuraches et al. 2012)	Minister of Health and Welfare publishes the final price to the public after review by the Health Insurance Policy Review Committee within the ministry (no other transparency protocols found)	No information found	Drugs that are already listed in the national formulary are scheduled for reassessment over 5 year period. Products that do not prove their clinical or economic value are to be eliminated from the list.	No information found	Social insurance program: Korean National Health Insurance (KNHI) funded by various sources, including premium, co-payment, tax, and employment funds and fee-for-service	No information found
Luxembourg (Caisse National de Sante 2011)	Decision documents are mailed to the applicant. If the decision is to not include the drug on the positive list, the statement of reasons based on objective and verifiable criteria are included in the decision.	If negative decision, the document includes information on the time and ways the owner can readdress the decision.	No information found	Decision to include the drug on a positive list shall take effect no earlier than the first day the second month following that in which the decision was notified to the holder.	No information found	Can readdress the decision: • Owner (manufacturer)
The Netherlands *General Reimbursement-Medicines Reimbursement*	Evaluation reports and/or assessments, recommendations, and minutes of meetings, publicly available on organization's website	No information found	<u>Extramural drugs:</u> • After 5 years, profits generated by a particular drug may be	<u>Extramural drugs:</u> • Special conditions exist depending on which reimbursement list the drug gets placed on (e.g., drug may	Private health insurance (every adult living in the Netherlands has the obligation to	None specified

System (GVS) (Stolk at al. 2009; Niezen et al. 2007; International Society for Pharmacoeconomics and Outcomes Research 2007)			reassessed (to prevent excessive profits stemming from market exclusivity) <u>Intramural drugs:</u> • After three years a reassessment takes place in order to assess whether listing/funding will continue • Reassessment criteria includes: therapeutic value, the actual costs of the medical product, the cost-effectiveness (based on data sources such as the clinical registration trials), and the efficient prescription	only be reimbursed for a small group of patients and must be prescribed by a specialist) <u>Intramural drugs:</u> • Treatment of all patients needs to be documented in a patient registry • Additional information needs to be collected through outcomes research • Some treatments are only covered if it is administered in combination with another drug	get basic health insurance)	

			(based on data collected through outcomes research in the Dutch clinical practice). This appraisal will decide if it will be included on the definitive listing/funding.			
New Zealand *General Reimbursement-Pharmaceutical Schedule* (PHARMAC 2013b; BPAC 2013; PHARMAC 2010)	The PHARMAC Pharmaceutical Schedule lists all the subsidized medications and conditions relating to their funding. This is available in hard copy (updated monthly) or online at: www.pharmac.govt.nz Copies of the Annual Review, Annual Report and Annual Plan, as well as information such as Pharmaceutical Schedule Updates, National Hospital Pharmaceutical Strategy, other publications and recent press releases are also available on the PHARMAC website.	There is no formal appeal mechanism for PHARMAC decisions; however, manufacturers may resubmit applications with additional information	Reviews of PHARMAC's funding decisions are done through a few mechanisms, namely: • Regular monitoring of the usage of medicines, including patient numbers and prescribing patterns; and • When required, reviews are carried out if	Pharmaceutical Schedule includes: • Pharmaceuticals available in the community • Some pharmaceuticals purchased by DHBs for use in their hospitals Drugs may be limited by indication or prescriber type	Community Pharmaceuticals (including cancer medicines delivered in a hospital setting) are funded by District Health Boards from the Combined Pharmaceutical Budget. Hospital medicines are funded by DHB hospitals.	Consultation letters about proposals to fund are sent to relevant patient groups, for example patient support groups. These letters are also published on the PHARMAC website and any member of the public can request to be included on the mailing list for consultation letters.

			new clinical information highlights efficacy or safety concerns with funded treatments If appropriate, PHARMAC would involve PTAC in the mechanisms above. Before any change is made to the listing of a treatment, PHARMAC would consult with affected parties as we would any proposal.			
New Zealand *Named Patient Pharmaceutical Assessment (Exceptional Circumstances) Policy (NPPA)* PHARMAC 2012; PHARMAC 2011; PHARMAC 2013a)	A notification of every funding decision made is issued and published on the PHARMAC website along with a basic summary of decisions made under the NPPA policy (pharmaceutical name, patient indication and funding decision). Not all NPPA applications are published on the website. As stated on the website – to protect patient confidentiality PHARMAC will not publish information about decisions where doing so could identify an individual patient (this could occur where there is only one person in New Zealand with a particular condition).	No formal appeal mechanism; PHARMAC will establish a review process for applicants not satisfied with decisions made under the NPPA Policy. Declined applications	All NPPA approvals are given for a limited time. Unless the applicant applied for a shorter period all initial approvals are given for 52 weeks. Evidence of ongoing benefit	Applications approved under the NPPA Policy may be for a limited time and renewals may need to meet conditions for continued funding. PHARMAC will advise the applicant of the duration of the approval (and therefore when an approval renewal application would need to be made) and of any conditions for	A funding provision for approved NPPA applications is made within the overall quantum provided for the Combined Pharmaceutical Budget.	None specified

		can be resubmitted any time if relevant new clinical circumstances arise or new evidence becomes available. PHARMAC will treat resubmitted applications as new applications.	is required on renewal.	continued funding		
Norway *General Reimbursement* (Festoy et al. 2011; Norwegian Medicines Agency 2013)	Norway has a reimbursement list regarding general reimbursement which is updated by NoMA once a month. The list of reimbursable medicines and associated criteria is published on the NoMA website as a searchable database	The applicant may appeal against decisions made by NoMA within three weeks of the date of the decision. If NoMA decides not to consent to the appeal, NoMA must submit the appeal to the Ministry, according to the Public Administration Act.	If a more cost-effective competitor is entering the market, the well-established medicine may become the second-line treatment. This will only take place after the company with the well-established medicine has had the opportunity to prove otherwise.	"Preferred Product": Norway has conditions set for reimbursement under the system of a preferred product. NoMA defines which of the therapeutically equivalent pharmaceuticals is preferred. The condition for reimbursement is that the preferred products are prescribed unless there is a valid medical reason for using other products.	National Insurance Administration is the payer, financed by taxation and state funding. Patients who are hospitalized with a rare disorder, the hospital itself is financially responsible for the administration of medicines. Hospitals are organized in five regions and are further	Appeal recommendations: • Applicant

					divided into hospital trusts. They have separate budgets and they are responsible for covering all use pharmaceuticals in the hospitals.	
Norway *Individual Reimbursement* Festoy et al. 2011	No information found	No information found	No information found	No information found	National Insurance Administration	No information found
Spain *General Reimbursement*	No information found	No information found	No information found	No information found	Insurance-based	No information found
Spain *Individual (case-by-case) or cohort reimbursement* (Garau and Mestre-Ferrandiz 2009; Whitfield et al. 2010)	**1) Temporary Use Authorization** Not an open process	In the case of a negative decision, the treating hospital may provide additional information within 10 days of the decision	No information found	No information found	Insurance-based	No information found
Spain *Individual (case-*	**2) Royal Decree 1015/2009** Not an open process	No information found	No information found	No information found	Insurance-based	No information found

by-case) or cohort reimbursement						
Spain *Individual (case-by-case) or cohort reimbursement* (Garau and Mestre-Ferrandiz 2009; Whitfield et al. 2010)	**3) Act 29/2006** No information found	No information found	No information found	No information found	Insurance-based	No information found
Sweden *General Reimbursement* (TLV 2012a; TLV 2012b; Moise and Docteur 2007; Anell and Persson 2005; Davidova et al. 2008)	• According to the principle of public access to official records, everybody has the right of access to public documents. However, this right is limited in certain cases (e.g. Protection of personal business and management relationships or other economic or person relationships). • Information is only kept from public access if there is a sufficient level of probability that an exposure of the information is harmful to a party. • Board meetings are not public and information discussed during these meeting is also not made public. • When the board makes a decision on a case, a statement is made in the minutes taken during the meeting. The minutes include info regarding if a decision has been made in a case, or if that matter requires a more detailed review. When the board makes a decision, it is reported separately. Thus, the content of the decision cannot be found in the minutes, but the minutes are published on the TLV website (www.tlv.se) generally one or two working days after the meeting. • Public decision are always released on the TLV website (www.tlv.se) once they have been signed and expedited.	• If a company does not agree with a decision on subsidization and price, the decision can be appealed against at the general administrative court. • The appeal must be sent to TLV and must be received within three weeks after the applicant received the decision.	• The TLV regularly reviews the reimbursement status of medicines to see if they should remain in the reimbursement system or not and can independently decide that a pharmaceutical or another product to be included in the pharmaceutical benefits should no longer be	Possible conditions include: • Requirement to provide supplementary data that shows an effect over long-term use • Demand for follow-up of use of the pharmaceutical in daily clinical routine • Requirements on the company's marketing	Orphan drugs are fully reimbursed through social insurance • The approved product is included on the national pharmaceutical benefits scheme (positive list) funded by the central government through income taxes • Government transfers funds to the county	Appeal recommendations: • Applicant

	• Documents used as a basis for decision by the TLV can be made public as soon as the decision is reported.	TLV then forwards the appeal to the court.	included. • Prior to commencing a new review the TLV makes detailed prioritization analyses where they, in part, look at sales volumes, reimbursement costs, cost per DDD, expanded indications and expired patents. The TLV also holds a dialogue with the county councils and provides them with an opportunity to submit suggestions on which products they should review.		councils	
Switzerland *General Reimbursement-*	Once the proceedings close, all interested parties have the right to view the documents, unless there are confidential data that could be harmful.	Applicant may appeal on grounds of evidence.	FOPH re-evaluates reimbursement of drugs every	Positive FOPH decision gets a new drug listed within 30 days after price agreement	Insurance-based When off-label	Appeal recommendations: • Applicant

List of Specialties (Office Federal de la Sante Publique 2013; International Society for Pharmacoeconomics and Outcomes Research 2011; Von Stoker et al. 2013)	Amendments are published in the Bulletin of the BAG in the first month after entry onto the LS	If a negative decision is projected, the FOPH informs the applicant, and the applicant may apply for revaluation with price adjustments or additional data.	three years and after patent expiry. Re-evaluation also takes place in case of new indications or changed limitations.		usage is reimbursed, they are either covered solely by the insurers or are divided between the insurer and the pharmaceutical company	Determine reimbursement amount for off-label/unauthorized products: Insurers
United Kingdom *General Reimbursement-Highly Specialised Technologies* (NICE 2014d, 2014a)	NICE produces three versions of its technology appraisals: the full appraisal presents the recommendations from • the full version in a format suited to implementation by health professionals and NHS bodies • the quick reference guide presents recommendations in a suitable format for health professionals information for the public is written for using suitable language for people without specialist medical knowledge	NICE has a standing Appeal Committee, which is appointed by the NICE Board. An appeal can be lodged by any of the appraisal consultees and can be considered at an oral hearing or by written submission. Consultees have 15 working days from the day the Final Appraisal Determination (FAD) is issued to make	When NICE publishes STA guidance, a review date is given. This is the month and year when NICE will consult with relevant organizations on a review proposal to decide whether or not the guidance needs to be updated, and if so, how to update the guidance. The length of time between guidance publication and the review date will vary	Some treatments are administered in designated hospitals only	National Health Service listing; primarily funded through general taxation	Appeal recommendations: • Manufacturers • Sponsors • Local/national groups representing patients & carers • Bodies representing health professionals • Department of Health Specialized commissioning groups

		an appeal. Grounds for appeals: • The Institute has failed to act fairly • The Institute formulated guidance which cannot reasonably be justified in the light of the evidence submitted • The Institute has exceeded its powers	depending on the available evidence for the drug, and knowledge of when ongoing research will be reported. NICE identifies new indications for the appraised drug, searches for new related technologies, assesses the progress of ongoing trials and gathers new available evidence.			
Scotland *General Reimbursement* (Scottish Medicines Consortium, 2013b, 2014a)	SMC publishes its advice on its website. All information received may be subject to disclosure under the Freedom of Information (Scotland) Act 2002. On receipt of a request for information, the SMC secretariat will contact the designated company representative to confirm that they are agreeable to the release of the information being requested and to give them the opportunity to identify information that is deemed as commercial in confidence. The Scottish Medicines Consortium (SMC) meetings are open to members of the public and press to observe. This supports SMC's commitment to openness and transparency. It enables stakeholders and members of the public to understand how evidence is assessed and interpreted, how consultation comments are taken into account and how recommendations are made.	If "not recommended" advice has been issued for a product, and the company wishes to consider a resubmission, they can request, via the Secretariat, a face-to-face meeting with SMC, with the aim of better	No information found	No information found	NHS Scotland; primarily funded through general taxation	Appeal recommendations: Applicant

		understanding why their product was not recommended, thus allowing an appropriate focus for the resubmission. Alternatively, where there is significant new information about a drug, or new analysis of existing information, the company may make a resubmission.				
Wales *General Reimbursement* (AWMSG 2014)	No information found	When the concerns of the applicant relate to differences in scientific opinion and/or interpretation of data, an Independent Review (IR) may be triggered, using the procedures set out below, by the applicant company: • A request for review	An advice review date may be set to ensure that additional clinical trial evidence or clinical audit data are reviewed, and this may require an additional submission	All patients receiving approved medicines should be entered into registries for recording prospective measures of clinical outcome	NHS Wales; primarily funded through general taxation	Appeal recommendations: Applicant

		will be submitted to AWTTC who will discuss the case with the Chairman of AWMSG. • In the event that it is agreed that there are substantial new data, the case shall in effect be treated as a new case and referred again to AWTTC and go through the normal AWMSG process once more. • In the event that any disagreement relating to the new data cannot be resolved, the Chairman of AWMSG				

		shall report the facts to the next meeting of AWMSG and establish a group to conduct an IR once it is satisfied that there are no preliminary issues relating to the substance of the appeal. • An IR panel will be appointed by AWMSG on advice from the Chairman and Secretariat of AWMSG. • The IR panel will review the original material considered by AWMSG				

		and also any new evidence submitted by the applicant. Should scientific support and advice be required then this shall be provided by AWTTC, but must be carried out by personnel not involved in the original review. • The IR panel will report back to AWMSG who will remain the final arbiter in all cases.				
United Kingdom *Innovation Pass* (UK Department of Health 2009)	There will be no communication in public as to which technologies have applied for an Innovation Pass. Information provided by the sponsor company for an Innovation Pass will be treated as "commercial in confidence" as its public disclosure could have an impact on the commercial interests of the company.	No appeal stage for Innovation Passes	Further applications for unsuccessful topics may be made	An important reason for the granting of an Innovation Pass is the collection of data that will inform the future NICE appraisal after the three years of the Pass. Although,	Funding takes place through the budget of the National Health Service	None specified

				national data collection through the granting of the Innovation Pass cannot be mandated, NHS bodies will be strongly encouraged to participate in data collection exercises organized by companies under the terms of their data collection plans.		
England & Wales *Patient Access Scheme* (NICE 2009b)	NICE will not put into the public domain any documents that are considered in the PASLU process. NICE considers it essential that schemes can be received and considered in confidence. NICE also understands that manufacturers may suffer commercial and other harm if information on the detail of proposed schemes were made publically available at this point. Therefore, NICE will treat all details of proposed schemes as confidential and will not release any information relating to it under the Freedom of Information Act or in any other circumstance, unless the manufacturer has agreed to the release.	No information found	Resubmissions because of significant changes to the outline of the patient access scheme will only be accepted after a renewed referral of the scheme by the Department of Health.	No information found	National Health Service	No information found
Scotland *Patient Access Scheme* (Scottish Medicines Consortium 2013a)	No information found	No information found	• If the PAS is not recommended, the company may resubmit a revised PAS. SMC/NICE will continue to assess the	Where a product with an accepted PAS is accepted for use or restricted use in NHS Scotland, the PASAG Secretariat will prepare an Implementation Pack to support implementation of the PAS by NHS Boards. The Implementation Pack will be issued to	National Health Service Scotland	No information found

			clinical and cost-effectiveness of medicines and PASAG will assess the acceptability of the PAS on behalf of NHS Scotland.	NHS Boards via SMC. Other than where the PAS is a simple discount scheme the Implementation Pack will include a PAS Monitoring Template to facilitate development of the PAS Monitoring Database by NHS Boards.		
Wales *Patient Access Scheme,* (AWMSG 2012b)	All information will be handled in accordance with the principles and requirements of the Data Protection Act and the Freedom of Information Act. WPAS details will be treated as confidential, and no documents will be disclosed or published in the public domain (unless the manufacturer has agreed to the release of information)	No information found	AWMSG final appraisal recommendations (FARs) will be reviewed three years after publication. At this stage, any Welsh Patient Access Schemes associated with a recommendation will also be subject to review	No information found	National Health Service	No information found