

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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Appendix A. Literature search strategy

TABLE 1. Weighted sample sizes of respondent disease cohorts

Search	Query
#85	Search #83 OR #84
#83	Search #17 AND #70 Filters: Publication date from 2004/01/01; Humans; English; French
#84	Search (#17 AND #70) AND (in process[sb] OR publisher[sb] OR pubmednotmedline[sb])
#71	Search #17 AND #70
#82	Search #17 AND #70 Filters: Publication date from 2004/01/01; English; French
#81	Search #17 AND #70 Filters: Publication date from 2004/01/01; English
#80	Search #17 AND #70 Filters: Publication date from 2004/01/01
#77	Search #17 AND (in process[sb] OR publisher[sb] OR pubmednotmedline[sb])
#17	Search #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #8 OR #14
#75	Search #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #8 OR #14 Filters: Publication date from 2004/01/01; Humans; English; French
#74	Search #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #8 OR #14 Filters: Publication date from 2004/01/01; Humans; English
#73	Search #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #8 OR #14 Filters: Publication date from 2004/01/01; Humans
#72	Search #1 OR #2 OR #3 OR #4 OR #5 OR #6 OR #8 OR #14 Filters: Publication date from 2004/01/01
#70	Search #46 OR #69
#69	Search #47 OR #48 OR #49 OR #50 OR #51 OR #52 OR #53 OR #54 OR #55 OR #56 OR #57 OR #58 OR #59 OR #62 OR #63 OR #64 OR #65 OR #66 OR #67 OR #68
#68	Search insurance[tj]
#67	Search budget*[tj]
#66	Search framework*[tj]
#65	Search regulat*[tj]
#64	Search legislat*[tj]
#63	Search HTA[tj]
#62	Search "technology assessment*[tj]

Table 2. Factors/criteria most commonly used by centralized drug review committees considering DRDs

Search	Query
#59	Search policy*[ti]
#58	Search policies[ti]
#57	Search policy[ti]
#56	Search decision*[ti]
#55	Search catastrophic[ti]
#54	Search "co-pay"*[ti]
#53	Search copay*[ti]
#52	Search cost-shar*[ti]
#51	Search access[ti]
#50	Search coverage[ti]
#49	Search fund*[ti]
#48	Search financ*[ti]
#47	Search reimburs*[ti]
#46	Search # 18 OR # 19 OR #20 OR #21 OR #22 OR #23 OR #24 OR #25 OR #26 OR #27 OR #28 OR #29 OR #30 OR #31 OR #32 OR #33 OR #34 OR #35 OR #36 OR #37 OR #38 OR #39 OR #40 OR #41 OR #42 OR #43 OR #44 OR #45
#45	Search ethics
#44	Search economics
#43	Search standards
#42	Search legislation and jurisprudence
#41	Search technology assessment, biomedical[mh]
#40	Search cost control[mh]
#39	Search cost sharing[mh]
#38	Search National Health Programs[mh]
#37	Search insurance, health[mh]
#36	Search insurance, health, reimbursement[mh]
#35	Search insurance coverage[mh]
#34	Search models, econometric[mh]
#33	Search models, economic[mh]
#32	Search economics[mh]

Table 2. Factors/criteria most commonly used by centralized drug review committees considering DRDs

Search	Query
#31	Search budgets[mh]
#30	Search moral obligations[mh]
#29	Search financing, organized[mh]
#28	Search cost-benefit analysis[mh]
#27	Search health services accessibility[mh]
#26	Search healthcare rationing[mh]
#25	Search delivery of healthcare[mh]
#24	Search reimbursement mechanisms[mh]
#23	Search state medicine[mh]
#22	Search public policy[mh]
#21	Search health policy[mh]
#20	Search policymaking[mh]
#19	Search decision-making, organizational[mh]
#18	Search decision-making[mh]
#14	Search "rare cancer**"[ti]
#8	Search "ultra rare"[ti]
#6	Search "orphan drug**"[ti]
#5	Search "orphan disease**"[ti]
#4	Search "rare disorder"[ti]
#3	Search "rare disease**"[ti]
#2	Search orphan drug production[mh]
#1	Search rare diseases[mh]

2. The Cochrane Library (issue 1 of 12, 2013, John Wiley and Sons)

Total results: 509 (0 relevant from 280 Cochrane Reviews, 6 from DARE, selected 1 from 172 Central trials, 0 relevant from 19 Methods, 19 from 19 from HTA, and 10 from 10 of NHS EED

#1 rare diseases:ti,ab,kw (Word variations have been searched)

#2 orphan drug production

#3 "rare disease**"

- #4 “rare disorder*”
- #5 “orphan disease*”
- #6 “orphan drug*”
- #7 “ultra rare”
- #8 “rare cancer*”
- #9 #1 or #2 or #3 or #4 or #5 or #6 or #7 or #8
- #10 decision-making
- #11 policymaking
- #12 health policy
- #13 public policy
- #14 state medicine
- #15 reimbursement mechanisms
- #16 delivery of healthcare
- #17 healthcare rationing
- #18 health services accessibility
- #19 cost-benefit analysis
- #20 financing, organized
- #21 moral obligations
- #22 economics
- #23 models, economic
- #24 models, econometric
- #25 insurance coverage
- #26 insurance, health
- #27 insurance, health, reimbursement
- #28 national health programs
- #29 cost sharing
- #30 cost control
- #31 technology assessment, biomedical
- #32 legislation and jurisprudence
- #33 standards
- #34 economics
- #35 ethics
- #36 #10 or #11 or #12 or #13 or #14 or #15 or #16 or #17 or #18 or #19 or #20 or #21 or #22 or #23 or #24 or #25 or #26 or #27 or #28 or #29 or #30 or #31 or #32 or #33 or #34 or #35
- #37 reimburs* or financ* or fund* or coverage or access* or cost* or copay* or “co-pay*” or catastrophic or decision*
- #38 #36 or #37
- #39 policy or policies or legislat* or regulat* or “technology assessment” or HTA or framework or budget or insurance
- #40 #38 or #39
- #41 #9 and #40

3. Centre for Reviews and Dissemination (CRD): DARE, NHS EED and HTA databases <http://www.crd.york.ac.uk/crdweb/HomePage.asp> (searched 28 Feb 2013)

1	MeSH DESCRIPTOR Rare Diseases EXPLODE ALL TREES
2	MeSH DESCRIPTOR Orphan Drug Production EXPLODE ALL TREES
3	("rare disease*") OR ("orphan drug*") OR ("ultra rare")
4	#1 OR #2 OR #3

4. EMBASE (Ovid, 2004 to 17 Feb 2013)

1	exp *rare disease/
2	exp *orphan drug/
3	ultra rare.mp.
4	1 or 2 or 3
5	exp *decision-making/
6	exp *healthcare policy/
7	exp *policy/
8	exp *national health service/
9	exp *economic aspect/
10	exp *reimbursement/ or exp *"healthcare cost"/ or exp *"cost"/
11	exp *healthcare delivery/
12	exp *"cost benefit analysis"/
13	exp *financial management/
14	exp *morality/
15	exp *ethics/ or exp *medical ethics/
16	exp *budget/
17	exp *economics/ or exp *health economics/
18	exp *health insurance/
19	exp *"cost-effectiveness analysis"/
20	exp *biomedical technology assessment/
21	exp *law/
22	exp *jurisprudence/
23	5 or 6 or 7 or 8 or 9 or 10 or 11 or 12 or 13 or 14 or 15 or 16 or 17 or 18 or 19 or 20 or 21 or 22
24	4 and 23
25	limit 24 to yr="1990 -Current"

5. Web of Science (Thomson Reuters, searched 13 Mar 2013)

#3	579 #2 AND #1 Timespan=2004-2013 Search language=English
#2	Topic=(decision* OR policy OR policies OR reimbursement OR rationing OR access OR accessibility) OR Topic=(financing OR economic* OR model* OR cost* OR assessment* OR budget*) OR Topic=(moral OR ethic* OR legislation) Timespan=2004-2013 Search language=English
#1	Title=("rare diseases" OR "rare disorder*" OR "orphan drug*" OR "orphan disease*" OR "ultra rare") Timespan=2004-2013 Search language=English

6. EconLit (EBSCOHost, searched 13 Mar 2013)

SI	rare disease* OR rare disorder* OR orphan drug* OR "ultra rare"	Limiters - Published Date from: 20040101-20131231 Search modes - Find all my search terms
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7. PAIS International (ProQuest, searched 14 Mar 2013)

(rare disease*) OR (rare disorder* OR orphan drug*) OR (ultra rare) limited 2004 to date

8. Sociological Abstracts (ProQuest, searched 14 Mar 2013)

(rare disease*) OR (rare disorder* OR orphan drug*) OR (ultra rare) limited 2004 to date

9. Canadian Business and Current Affairs (CBCA Complete, Proquest, searched 20 Mar 2013)

ti(rare disease*) OR ti((rare disorder* OR orphan drug*)) OR ti(ultra rare) limited 2004 to date, English or French

10. ABI/INFORM Global (Proquest, searched 20 Mar 2013)

ti((rare disease*) OR (rare disorder*) OR (orphan drug*) OR (ultra rare)) limited 1990 to date, English or French

11. Scopus (SciVerse, searched 20 Mar 2013)

(TITLE("rare disease*" OR "rare disorder*" OR "orphan drug*" OR "ultra rare") AND PUBYEAR > 1989) AND (TITLE(decision* OR policy* OR policies OR reimbursement OR delivery OR rationing OR access* OR financing OR economic* OR coverage OR cost* OR legislation* OR funding))

12. Proquest Dissertations and Theses Fulltext (Proquest, searched 20 Mar 2013)

all((rare disease*) OR (rare disorder*) OR (orphan drug*) OR (ultra rare)) AND all(decision* OR policy OR policies OR reimbursement OR rationing OR access* OR economic* OR funding OR legislation OR coverage) limited 1990 to date, English or French

13. Canadian Newsstand Complete (Proquest, searched 20 Mar 2013)

ti((rare disease*) OR (rare disorder*) OR (orphan drug*) OR (ultra rare)) AND ti(decision* OR policy OR policies OR reimbursement OR economic* OR rationing OR access* OR fund* OR legislation OR catastrophic OR regular*) limited to 1990 to date, English or French, document type: Article, Bibliography, Book, Commentary, Conference, Editorial, Essay, Feature, General Information, Government and Official Document, Review

Grey literature search (searched April 2013; *unless otherwise noted, the search terms were: rare disease* or rare disorder* or orphan drug*)

Canada

- KU-UC (Quebec Population Health Research Network (QPHRN)) <http://www.santepop.qc.ca/en/index.html>
- Canadian Organization for Rare Disorders <http://www.raredisorders.ca/> *scanned web site
- National Library of Canada. AMICUS: Canadian National Catalogue <http://www.collectionscanada.gc.ca/amicus/index-e.html>

US

- New York Academy of Medicine Grey literature collection <http://www.greylit.org/home>
- RAND www.rand.org
- US Food and Drug Administration. Rare Diseases Program <http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm221248.htm> *scanned web page and publications
- US Food and Drug Administration. Humanitarian Device Exemptions <http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/PremarketSubmissions/HumanitarianDeviceExemption/default.htm> *scanned web page
- National Institutes of Health Office of Rare Diseases Research <http://rarediseases.info.nih.gov/> *scanned web page and resources
- National Organization for Rare Disorders (NORD) www.rarediseases.org *scanned web site and publications
- Patient-Centred Outcomes Research Institute <http://www.pcori.org/> *scanned web page

Europe

- NHS Evidence www.evidence.nhs.uk *rare diseases in their filter categories: management, commissioning, policy and service development
- Open Grey <http://www.opengrey.eu/>
- Rare Cancers Foundation <http://www.rarercancers.org.uk/> *scanned policy section / reports
- Genetic Alliance UK <http://www.geneticalliance.org.uk/> *scanned publications
- Orphanet: the portal for rare diseases and orphan drugs <http://www.orpha.net/consor/cgi-bin/index.php> *scanned web page sections and 2012-2013 issues of newsletter
- EURORDIS: Rare Diseases Europe <http://www.eurordis.org/about-eurordis>

- European Commission http://ec.europa.eu/index_en.htm *scanned web page on health / human diseases / policy
- European Union Committee of Experts on Rare Diseases http://www.eucerd.eu/?page_id=13 *scanned web page sections on recommendations / reports / and national resources
- http://www.eucerd.eu/?page_id=154 *scanned country-by-country publications

Australia

- Rare Voices Australia <http://www.rarevoices.org.au/> *scanned web page
- Australian Government. Life Saving Drugs Program <http://www.health.gov.au/lscp> *scanned web