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AVIS

Glassia^{MC} – Déficit congénital en
alpha₁-antitrypsine
Annexes complémentaires

Une production de l'Institut national
d'excellence en santé
et en services sociaux (INESSS)

Direction de l'évaluation des médicaments et
des technologies à des fins de remboursement

Le présent document contient les annexes complémentaires au rapport d'évaluation d'un produit du système du sang Glassia^{MC} – Déficit congénital en alpha₁-antitrypsine pour la mise à jour de la *Liste des produits du système du sang du Québec*.

Le contenu de cette publication a été rédigé et édité par l'INESSS.

Ces annexes et le rapport final sont accessibles en ligne dans la section [Publications](#) de notre site Web.

Renseignements

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Responsabilité

L'Institut rend accessibles les principales informations qui ont servi à la préparation du rapport Évaluation d'un produit du système du sang pour la mise à jour de la *Liste des produits du système du sang du Québec* aux lecteurs et lectrices qui désirent plus de détails sur sa démarche scientifique.

Ce document n'a pas fait l'objet d'une révision linguistique. Il ne reflète pas forcément les opinions des autres personnes consultées aux fins du présent dossier.

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ANNEXE A

Décisions d'autres juridictions

Tableau A-1 Recommandations des autres organismes et agences

| Organismes/ Agences* | En cours d'évaluation | Évalué | | Particularités |
|---------------------------------|--------------------------|-----------------|-----------------|---|
| | | Avis positif | Avis négatif | |
| Mise en marché | | | | |
| FDA ¹ | | X | | Treatment of chronic augmentation and maintenance therapy in individuals with emphysema due to congenital deficiency of alpha-1-proteinase inhibitor (Alpha1-PI), also known as alpha1-antitrypsin. |
| EMA | | | | Aucune évaluation |
| Recommandations – remboursement | | | | |
| NICE | | | | Aucune évaluation |
| HAS | | X | | Aucune évaluation |
| Scottish/Wales | | | | Aucune évaluation |
| Australie | | | | Aucune évaluation |
| ACMTS | | | | Aucune évaluation |
| SwissMedic | | | | Aucune évaluation |

* En date du 16 février 2023.

¹ Food and Drug Administration (FDA). Glassia [site Web]. Disponible au : <https://www.fda.gov/vaccines-blood-biologics/approved-blood-products/glassia> (consulté le 16 février 2023).

ANNEXE B

Stratégie de repérage de l'information scientifique

Tableau B-1 Bases de données bibliographiques

| MEDLINE(R) and Epub Ahead of Print, In-Process, In-Data-Review & Other Non-Indexed Citations, Daily and Versions <1946 to January 12, 2023> (Ovid) Date du repérage : 13 janvier 2023 Limites : anglais, français; 1987-2023, humains | |
|---|---|
| 1 | alpha 1-Antitrypsin/ |
| 2 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).ti,ab,kf,ot,hw,rn,nm. |
| 3 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ4 (protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits)).ti,ab,kf,ot,hw,rn,nm. |
| 4 | (alpha-1-PI OR alfa-1-PI OR Alpha1-PI OR alfa1-PI OR alpha-one-PI OR alfa-one-PI).ti,ab,kf,ot,hw,rn,nm. |
| 5 | (A1PI OR A1-PI OR A1AT OR alfa1astin* OR Alpha1AT OR aralast* OR csl-964* OR csl964* OR F431396OIS OR glassia* OR IA1P OR IA1-P OR infinia OR prolantin* OR pulmolast* OR respitin* OR respikam* OR respreeza* OR serpina1 OR serpin-A1 OR trypson* OR zemaira*).ti,ab,kf,ot,hw,rn,nm. |
| 6 | ((antitrypsin? OR anti-trypsin?) ADJ2 Pittsburgh).ti,ab,kf,ot,hw,rn,nm |
| 7 | OR/1-6 |
| 8 | alpha 1-Antitrypsin Deficiency/ |
| 9 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).ti,ab,kf. |
| 10 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 ((protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits))).ti,ab,kf. |
| 11 | (A1AT OR A1AP OR A1A OR AAT OR AATs).ti,ab,kf. |
| 12 | (default OR deficiencies OR deficiency OR deficient OR deficit? OR depriv* OR lack*).ti,ab,kf. |
| 13 | (9 OR 10 OR 11) ADJ5 12 |
| 14 | (mckusick-10740 OR A1AD OR AATD OR A1ATD OR A-1ATD).ti,ab,kf. |
| 15 | (genotyp* ADJ2 (PiZZ OR "PiZ(null)" OR "PiZ (null)" OR "Pi(null,null)" OR "Pi (null,null)" OR PiSZ)).ti,ab,kf. |
| 16 | 8 OR 13 OR 14 OR 15 |
| 17 | Pulmonary Emphysema/ |
| 18 | emphysema*.ti,ab,kf. |
| 19 | ((lung? OR pulmonary) ADJ3 (deficiencies OR deficiency OR deficient OR deficit?)).ti,ab,kf. |
| 20 | (chronic obstructive pulmonary ADJ (disease* OR disorder*)).ti,ab,kf. |
| 21 | (COPD OR COPDs).ti,kf. |
| 22 | OR/17-21 |
| 23 | 7 AND 16 |
| 24 | 22 AND 23 |
| 25 | ..!24 lg=English OR lg=French |
| 26 | ..!25 yr=1987-2023 |
| 27 | 26 NOT (animals/ NOT humans/) |
| 28 | ec.fs. OR Decision Trees/ OR exp Economics/ OR Likelihood Functions/ OR Linear Models/ OR Logistic Models/ OR Markov Chains/ OR Models, Economic/ OR Models, Econometric/ OR Models, Statistical/ OR Monte Carlo Method/ OR Nomograms/ OR Proportional Hazards Models/ |
| 29 | (afford* OR allocat* OR amounts OR budget* OR charge OR charged OR charging OR cheap* OR compensat* OR contract* OR cost OR costs OR cost-analys* OR cost-based OR cost-benefit* OR cost-effect* OR cost-eficc* OR debt* OR deficit* OR economic* OR equity OR equities OR expenditure* OR expens* OR fair* OR fee OR fees OR financ* OR fiscal* OR funding* OR high-cost* OR high-income* OR income* OR inequalit* OR inequit* OR invested OR investing OR investment* OR low-cost* OR low-income* OR Markov* OR money OR monetary OR nonexpens* OR non-expens* OR onerous* OR pay OR payment* OR payer* OR paying OR pharmaco-economic* OR pharmaco-economic* OR poverty OR price* OR pricing OR (public ADJ1 private ADJ1 (associat* OR collabor* OR partner*)) OR purchas* OR rationing OR reimburs* OR saving* OR socioeconomic* OR socio-economic* OR tax OR taxes OR taxing OR taxpayer* OR unafford*).ti,ab,kf. |
| 30 | ((clinical OR critical OR inpatient* OR in-patient* OR medical OR outpatient* OR out-patient* OR patient*) ADJ1 (path\$1 OR pathway* OR path-way*)) OR copay OR copayer* OR copaying OR copayment* OR co-pay OR co-payment* OR co-payer* OR co-paying OR (decision ADJ2 (tree* OR analys* OR model*)) OR discount* OR ((federal* OR state* OR |

| MEDLINE(R) and Epub Ahead of Print, In-Process, In-Data-Review & Other Non-Indexed Citations, Daily and Versions <1946 to January 12, 2023> (Ovid) Date du repérage : 13 janvier 2023 Limites : anglais, français; 1987-2023, humains | |
|--|--|
| | public* OR government*) ADJ2 funded) OR ((increas* OR improv* OR more) ADJ1 access*) OR marginal analys* OR Monte Carlo* OR sensitivity analys* OR (statistic* ADJ2 model*) OR "willingness to pay").ti,ab,kf. |
| 31 | OR/28-30 |
| 32 | 27 AND 31 |
| 33 | (Adaptive Clinical Trial OR Clinical Study OR Clinical Trial OR Clinical Trial, Phase I OR Clinical Trial, Phase II OR Clinical Trial, Phase III OR Clinical Trial, Phase IV OR Controlled Clinical Trial OR Clinical Trial Protocol OR Equivalence Trial OR Observational Study OR Pragmatic Clinical Trial OR Randomized Controlled Trial).pt. |
| 34 | Clinical Studies as Topic/ OR Clinical Trials as Topic/ OR Adaptive Clinical Trials as Topic/ OR Clinical Trials, Phase I as Topic/ OR Clinical Trials, Phase II as Topic/ OR Clinical Trials, Phase III as Topic/ OR Clinical Trials, Phase IV as Topic/ OR Controlled Clinical Trials as Topic/ OR Control Groups/ OR Double-Blind Method/ OR Equivalence Trials as Topic/ OR Intention to Treat Analysis/ OR Non-Randomized Controlled Trials as Topic/ OR Observational Studies as Topic/ OR Placebos/ OR Pragmatic Clinical Trials as Topic/ OR Random Allocation/ OR Randomized Controlled Trials as Topic/ OR Single-Blind Method/ OR Single-Case Studies as Topic/ |
| 35 | Registries/ OR Pharmacovigilance/ OR Product Surveillance, Postmarketing/ |
| 36 | (nonrandom* OR non-random* OR placebo* OR quasirandom* OR quasi-random* OR sham).ti,ab,hw,kf,kw. |
| 37 | ((doubl* OR singl* OR trebl* OR tripl*) ADJ (blind* OR dumm* OR mask*)).ti,ab,hw,kf,kw. |
| 38 | ((control* OR single-arm) ADJ3 (group? OR studies OR study OR trial?)).ti,ab,hw,kf,kw. |
| 39 | allocated.ti,ab,hw,kf,kw. |
| 40 | ((openlabel OR open-label) ADJ5 (studies OR study OR trial?)).ti,ab,hw,kf,kw. |
| 41 | ((equivalence OR noninferiority OR non-inferiority OR superiority) ADJ3 (studies OR study OR trial?)).ti,ab,hw,kf,kw. |
| 42 | (pragmatic ADJ (studies OR study)).ti,ab,hw,kf,kw. |
| 43 | ((practical OR pragmatic) ADJ3 trial*).ti,ab,hw,kf,kw. |
| 44 | ((quasixperimental OR quasi-experimental) ADJ3 (studies OR study OR trial?)).ti,ab,hw,kf,kw. |
| 45 | (phase ADJ3 (I OR "1" OR II OR "2" OR III OR "3" OR IV OR "4") ADJ3 (studies OR study OR trial?)).ti,ab,hw,kf,kw. |
| 46 | (information system\$1 OR records OR register\$1 OR registry OR registries).ti,ab,hw,kf,kw. |
| 47 | (real-world ADJ2 evidence*).ti,ab,hw,kf,kw. |
| 48 | (postcommercial#ation OR post-commercial#ation OR postmarket* OR post-market*).ti,ab,hw,kf,kw. |
| 49 | (pharmacovigilan* OR pharmaco-vigilan* OR (drug\$1 ADJ3 (monitoring OR reporting OR surveillance))).ti,ab,hw,kf,kw. |
| 50 | OR/33-49 |
| 51 | 27 AND 50 |
| 52 | ae.fs. OR Algorithms/ OR Comparative Effectiveness Research/ OR Decision Making/ OR Drug Approval/ OR Drug Development/ OR exp "Drug-Related Side Effects and Adverse Reactions"/ OR Efficiency, Organizational/ OR Efficiency/ OR "Evaluation Studies as Topic"/ OR Evaluation Study/ OR Evidence-Based Practice/ OR Forced Expiratory Volume/de OR Functional Residual Capacity/de OR Government Regulation/ OR Outcome Assessment, Health Care/ OR Practice Guidelines as Topic/ OR Program Evaluation/ OR Quality of Life/ OR Quality-Adjusted Life Years/ OR Risk Factors/ OR Total Lung Capacity/de OR Treatment Outcome/ |
| 53 | (accomplish* OR achiev* OR assess* OR attain* OR barrier* OR best OR consequen* OR decreas* OR effect* OR efficac* OR efficien* OR enhanc* OR established OR evaluat* OR evidence* OR fail* OR fulfil* OR guidance* OR guideline* OR guide-line* OR guiding OR hypersensitiv* OR hypersensitiv* OR immunogeni* OR immuno-geni* OR impact* OR improv* OR increas* OR ineffect* OR ineffic* OR influenc* OR insuccess\$2 OR (life ADJ2 quality) OR negative* OR nonfulfillment\$1 OR nonperform* OR nonsuccess\$2 OR outcome* OR out-come* OR perform* OR ((patient* OR personal) ADJ2 (satisfaction OR autonomy)) OR positive* OR posttest* OR post-test* OR pretest* OR pre-test* OR prevent* OR promising OR proven OR recommend* OR reduc* OR satisf* OR standard* OR succeed* OR success* OR tolerab* OR unachiev* OR underperform* OR unfulfill* OR unimprov* OR unmet OR unperform* OR unsuccess* OR validated).ti,ab,kf. |
| 54 | ((adverse OR dangerous OR harmful OR indirect OR injurious OR secondary OR serious OR side OR undesirable) ADJ1 (complication* OR event* OR reaction*)).ti,ab,kf. |
| 55 | OR/52-54 |
| 56 | 27 AND 55 |
| 57 | 51 OR 56 |
| 58 | 32 OR 57 |

| EMBASE <1974 to 2023 January 12> (Ovid) | |
|--|---|
| Date du repérage : 13 janvier 2023 | |
| Limites : anglais, français; 1987-2023, humains | |
| 1 | alpha 1-Antitrypsin/ |
| 2 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).ti,ab,kf,ot,hw,rn,dy. |
| 3 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ4 (protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits)).ti,ab,kf,ot,hw,rn,dy. |
| 4 | (alpha-1-PI OR alfa-1-PI OR Alpha1-PI OR alfa1-PI OR alpha-one-PI OR alfa-one-PI).ti,ab,kf,ot,hw,rn,dy. |
| 5 | (A1PI OR A1-PI OR A1AT OR alfalastin* OR Alpha1AT OR aralast* OR csl-964* OR csl964* OR F431396OIS OR glassia* OR IA1P OR IA1-P OR infinia OR prolastin* OR pulmolast* OR respitin* OR respikam* OR respreeza* OR serpina1 OR serpin-A1 OR trypson* OR zemaira*).ti,ab,kf,ot,hw,rn,dy. |
| 6 | ((antitrypsin? OR anti-trypsin?) ADJ2 Pittsburgh).ti,ab,kf,ot,hw,rn,dy. |
| 7 | OR/1-6 |
| 8 | alpha 1-Antitrypsin Deficiency/ |
| 9 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).ti,ab,kf. |
| 10 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 ((protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits))).ti,ab,kf. |
| 11 | (A1AT OR A1AP OR A1A OR AAT OR AATs).ti,ab,kf. |
| 12 | (default OR deficiencies OR deficiency OR deficient OR deficit? OR depriv* OR lack*).ti,ab,kf. |
| 13 | (9 OR 10 OR 11) ADJ5 12 |
| 14 | (mckusick-10740 OR A1AD OR AATD OR A1ATD OR A-1ATD).ti,ab,kf. |
| 15 | (genotyp* ADJ2 (PiZZ OR "PiZ(null)" OR "PiZ (null)" OR "Pi(null,null)" OR "Pi (null,null)" OR PiSZ)).ti,ab,kf. |
| 16 | 8 OR 13 OR 14 OR 15 |
| 17 | Lung Emphysema/ |
| 18 | emphysema*.ti,ab,kf. |
| 19 | ((lung? OR pulmonary) ADJ3 (deficiencies OR deficiency OR deficient OR deficit?)).ti,ab,kf. |
| 20 | (chronic obstructive pulmonary ADJ (disease* OR disorder*)).ti,ab,kf. |
| 21 | (COPD OR COPDs).ti,kf. |
| 22 | OR/17-21 |
| 23 | 7 AND 16 |
| 24 | 22 AND 23 |
| 25 | ..//24 lg=English OR lg=French |
| 26 | ..//25 yr=1987-2023 |
| 27 | 26 NOT ((nonhuman/ OR animal/ NOT human/) |
| 28 | (de OR pe).fs. OR Econometric Model/ OR exp Economic Aspect/ OR exp Markov Chain/ OR Markov Chain Monte Carlo Method/ OR Monte Carlo Method/ OR Statistical Model/ |
| 29 | (afford* OR allocat* OR amounts OR budget* OR charge OR charged OR charging OR cheap* OR compensat* OR contract* OR cost OR costs OR cost-analys* OR cost-based OR cost-benefit* OR cost-effect* OR cost-eflic* OR debt* OR deficit* OR economic* OR equity OR equities OR expenditure* OR expens* OR fair* OR fee OR fees OR financ* OR fiscal* OR funding* OR high-cost* OR high-income* OR income* OR inequalit* OR inequit* OR invested OR investing OR investment* OR low-cost* OR low-income* OR Markov* OR money OR monetary OR nonexpens* OR non-expens* OR onerous* OR pay OR payer* OR paying OR pharmaco-economic* OR pharmaco-economic* OR poverty OR price* OR pricing OR (public ADJ1 private ADJ1 (associat* OR collabor* OR partner*)) OR purchas* OR rationing OR reimburs* OR saving* OR socioeconomic* OR socio-economic* OR tax OR taxes OR taxing OR taxpayer* OR unafford*).ti,ab,kf. |
| 30 | ((((clinical OR critical OR inpatient* OR in-patient* OR medical OR outpatient* OR out-patient* OR patient*) ADJ1 (path\$1 OR pathway* OR path-way*)) OR copay OR copayer* OR copaying OR copayment* OR co-pay OR co-payment* OR co-payer* OR co-paying OR (decision ADJ2 (tree* OR analys* OR model*)) OR discount* OR ((federal* OR state* OR public* OR government*) ADJ2 funded) OR ((increas* OR improv* OR more) ADJ1 access*) OR marginal analys* OR Monte Carlo* OR sensitivity analys* OR (statistic* ADJ2 model*) OR "willingness to pay")).ti,ab,kf. |
| 31 | OR/28-30 |
| 32 | 27 AND 31 |
| 33 | Adaptive Clinical Trial/ OR Case Control Study/ OR Case Report/ OR Case Study/ OR Clinical Article/ OR Clinical Study/ OR Clinical Trial/ OR Clinical Trial Protocol/ OR Community Trial/ OR Control Group/ OR Controlled Clinical Trial/ OR Double Blind Procedure/ OR Equivalence Trial/ OR Family Study/ OR Hospital Based Case Control Study/ OR Intention to Treat Analysis/ OR Intervention Study/ OR Longitudinal Study/ OR Major Clinical Study/ OR Multicenter Study/ OR Non-Inferiority Trial/ OR Observational Study/ OR Open Study/ OR Phase 1 Clinical Trial/ OR Phase 2 Clinical Trial/ OR Phase 3 Clinical Trial/ OR Phase 4 Clinical Trial/ OR Placebo/ OR Population Based Case Control Study/ OR Pragmatic |

| EMBASE <1974 to 2023 January 12> (Ovid) | |
|--|---|
| Date du repérage : 13 janvier 2023 | |
| Limites : anglais, français; 1987-2023, humains | |
| | Trial/ OR Prospective Study/ OR Randomization/ OR Randomized Controlled Trial/ OR Retrospective Study/ OR Single Blind Procedure/ OR Single-Case Study/ OR Superiority Trial/ |
| 34 | Cause of Death Registry/ OR Chronic Disease Registry/ OR Clinical Trial Registry/ OR Death Registry/ OR Disease Registry/ OR Medical Device Registry/ OR Patient Registry/ OR Procedure Registry/ OR Register/ |
| 35 | Drug Surveillance Program/ OR Pharmacovigilance/ OR Postmarketing Surveillance/ OR Special Situation for Pharmacovigilance/ OR Unexpected Outcome of Drug Treatment/ |
| 36 | ((nonrandom* OR non-random* OR placebo* OR quasirandom* OR quasi-random* OR random* OR sham).ti,ab,hw,kf,kw. |
| 37 | ((doubl* OR singl* OR trebl* OR tripl*) ADJ (blind* OR dumm* OR mask*).ti,ab,hw,kf,kw. |
| 38 | ((control* OR single-arm) ADJ3 (group? OR studies OR study OR trial?).ti,ab,hw,kf,kw. |
| 39 | allocated.ti,ab,hw,kf,kw. |
| 40 | ((openlabel OR open-label) ADJ5 (studies OR study OR trial?).ti,ab,hw,kf,kw. |
| 41 | ((equivalence OR noninferiority OR non-inferiority OR superiority) ADJ3 (studies OR study OR trial?).ti,ab,hw,kf,kw. |
| 42 | (pragmatic ADJ (studies OR study)).ti,ab,hw,kf,kw. |
| 43 | ((practical OR pragmatic) ADJ3 trial*).ti,ab,hw,kf,kw. |
| 44 | ((quasixperimental OR quasi-experimental) ADJ3 (studies OR study OR trial?).ti,ab,hw,kf,kw. |
| 45 | (phase ADJ3 (I OR "1" OR II OR "2" OR III OR "3" OR IV OR "4")) ADJ3 (studies OR study OR trial?).ti,ab,hw,kf,kw. |
| 46 | (information system\$1 OR records OR register\$1 OR registry OR registries).ti,ab,hw,kf,kw. |
| 47 | (real-world ADJ2 evidence*).ti,ab,hw,kf,kw. |
| 48 | (postcommercial#ation OR post-commercial#ation OR postmarket* OR post-market*).ti,ab,hw,kf,kw. |
| 49 | (pharmacovigilan* OR pharmaco-vigilan* OR (drug\$1 ADJ3 (monitoring OR reporting OR surveillance))).ti,ab,hw,kf,kw. |
| 50 | OR/33-49 |
| 51 | 27 AND 50 |
| 52 | ae.fs. OR exp Adverse Drug Reaction/ OR exp Algorithm/ OR Comparative Effectiveness/ OR Comparative Study/ OR Consensus Development/ OR exp Decision Making/ OR exp Drug Approval/ OR exp Drug Development/ OR Evaluation Study/ OR Evidence Based Emergency Medicine/ OR Evidence-Based Medicine/ OR Evidence-Based Pharmacy/ OR Evidence-Based Practice/ OR Evidence-Based Practice Center/ OR Forced Expiratory Volume/ OR Functional Residual Capacity/ OR Government Regulation/ OR GRADE Approach/ OR Organizational Efficiency/ OR Outcome Assessment/ OR Outcomes Research/ OR exp Practice Guideline/ OR Productivity/ OR exp Program Evaluation/ OR Quality Adjusted Life Year/ OR "Quality of Life"/ OR Risk Factor/ OR Total Lung Capacity/ OR exp Treatment Outcome/ |
| 53 | ((accomplish* OR achiev* OR assess* OR attain* OR barrier* OR best OR consequen* OR decreas* OR effect* OR efficac* OR efficien* OR enhanc* OR established OR evaluat* OR evidence* OR fail* OR fulfil* OR guidance* OR guideline* OR guide-line* OR guiding OR hypersensitiv* OR hypersensitiv* OR immunogeni* OR immuno-geni* OR impact* OR improv* OR increas* OR ineffect* OR ineffic* OR influenc* OR insuccess\$2 OR (life ADJ2 quality) OR negative* OR nonfulfillment\$1 OR nonperform* OR nonsuccess\$2 OR outcome* OR out-come* OR perform* OR ((patient* OR personal) ADJ2 (satisfaction OR autonomy)) OR positive* OR posttest* OR post-test* OR pretest* OR pre-test* OR prevent* OR promising OR proven OR recommend* OR reduc* OR satisf* OR standard* OR succeed* OR success* OR tolerab* OR unachiev* OR underperform* OR unfulfill* OR unimprov* OR unmet OR unperform* OR unsuccess* OR validated).ti,ab,kf. |
| 54 | ((adverse OR dangerous OR harmful OR indirect OR injurious OR secondary OR serious OR side OR undesirable) ADJ1 (complication* OR event* OR reaction*).ti,ab,kf. |
| 55 | OR/52-54 |
| 56 | 27 AND 55 |
| 57 | 51 OR 56 |
| 58 | 32 OR 57 |

| EBM Reviews (Ovid) – Cochrane Database of Systematic Reviews 2005 to January 10, 2023 | |
|--|--|
| Date du repérage : 13 janvier 2023 | |
| Limites : anglais, français; 1987-2023, humains | |
| 1 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?).ti,ab,kw. |
| 2 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ4 (protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits)).ti,ab,kw. |
| 3 | (alpha-1-PI OR alfa-1-PI OR Alpha1-PI OR alfa1-PI OR alpha-one-PI OR alfa-one-PI).ti,ab,kw. |
| 4 | (A1PI OR A1-PI OR A1AT OR alfalastin* OR Alpha1AT OR aralast* OR csl-964* OR csl964* OR F431396OIS OR glassia* OR IA1P OR IA1-P OR infinia OR prolastin* OR pulmolast* OR respitin* OR respikam* OR respreeza* OR serpina1 OR serpin-A1 OR trypson* OR zemaira*).ti,ab,kw. |
| 5 | ((antitrypsin? OR anti-trypsin?) ADJ2 Pittsburgh).ti,ab,kw. |

| EBM Reviews (Ovid) – Cochrane Database of Systematic Reviews 2005 to January 10, 2023 | |
|--|--|
| Date du repérage : 13 janvier 2023 | |
| Limites : anglais, français; 1987-2023, humains | |
| 6 | OR/1-5 |
| 7 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).ti,ab,kw. |
| 8 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 ((protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits))).ti,ab,kw. |
| 9 | (A1AT OR A1AP OR A1A OR AAT OR AATs).ti,ab,kw. |
| 10 | (default OR deficiencies OR deficiency OR deficient OR deficit? OR depriv* OR lack*).ti,ab,kw. |
| 11 | (7 OR 8 OR 9) ADJ5 10 |
| 12 | (mckusick-10740 OR A1AD OR AATD OR A1ATD OR A-1ATD).ti,ab,kw. |
| 13 | (genotyp* ADJ2 (PiZZ OR "PiZ(null)" OR "PiZ (null)" OR "Pi(null,null)" OR "Pi (null,null)" OR PiSZ)).ti,ab,kw. |
| 14 | 11 OR 12 OR 13 |
| 15 | emphysema*.ti,ab,kw. |
| 16 | ((lung? OR pulmonary) ADJ3 (deficiencies OR deficiency OR deficient OR deficit?)).ti,ab,kw. |
| 17 | (chronic obstructive pulmonary ADJ (disease* OR disorder*)).ti,ab,kw. |
| 18 | (COPD OR COPDs).ti,kw. |
| 19 | OR/15-18 |
| 20 | 6 AND 14 |
| 21 | 19 AND 20 |
| 22 | ..!/21 yr=1987-2023 |

| EBM Reviews (Ovid) – Health Technology Assessment 4th Quarter 2016 | |
|--|--|
| EBM Reviews (Ovid) – NHS Economic Evaluation Database 1st Quarter 2016 | |
| Date du repérage : 13 janvier 2023 | |
| Limites : 1987-2023, humains | |
| 1 | alpha 1-Antitrypsin/ |
| 2 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).tw. |
| 3 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ4 (protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits)).tw. |
| 4 | (alpha-1-PI OR alfa-1-PI OR Alpha1-PI OR alfa1-PI OR alpha-one-PI OR alfa-one-PI).tw. |
| 5 | (A1PI OR A1-PI OR A1AT OR alfa1astin* OR Alpha1AT OR aralast* OR csl-964* OR csl964* OR F431396OIS OR glassia* OR IA1P OR IA1-P OR infinia OR prolantin* OR pulmolast* OR respitin* OR respikam* OR respreeza* OR serpina1 OR serpin-A1 OR trypson* OR zemaira*).tw. |
| 6 | ((antitrypsin? OR anti-trypsin?) ADJ2 Pittsburgh).tw. |
| 7 | OR/1-6 |
| 8 | alpha 1-Antitrypsin Deficiency/ |
| 9 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 (antiprotease? OR anti-protease? OR antiproteinase? OR anti-proteinase? OR antitrypsin? OR anti-trypsin?)).tw. |
| 10 | ((alfa-1 OR alfa1 OR alfa-one OR alpha-1 OR alpha1 OR alpha-one OR a1) ADJ3 ((protease? OR proteinase? OR trypsin?) ADJ4 (inhibitor* OR inhibit OR inhibits))).tw. |
| 11 | (A1AT OR A1AP OR A1A OR AAT OR AATs).tw. |
| 12 | (default OR deficiencies OR deficiency OR deficient OR deficit? OR depriv* OR lack*).tw. |
| 13 | (9 OR 10 OR 11) ADJ5 12 |
| 14 | (mckusick-10740 OR A1AD OR AATD OR A1ATD OR A-1ATD).tw. |
| 15 | (genotyp* ADJ2 (PiZZ OR "PiZ(null)" OR "PiZ (null)" OR "Pi(null,null)" OR "Pi (null,null)" OR PiSZ)).tw. |
| 16 | 8 OR 13 OR 14 OR 15 |
| 17 | Pulmonary Emphysema/ |
| 18 | emphysema*.tw. |
| 19 | ((lung? OR pulmonary) ADJ3 (deficiencies OR deficiency OR deficient OR deficit?)).tw. |
| 20 | (chronic obstructive pulmonary ADJ (disease* OR disorder*)).tw. |
| 21 | (COPD OR COPDs).tw. |
| 22 | OR/17-21 |
| 23 | 7 AND 16 |
| 24 | 22 AND 23 |
| 25 | ..!/24 yr=1987-2023 |

Tableau B-2 Critères PICOTS considérés pour la recherche documentaire

| Paramètres | Critères d'inclusion | Critères d'exclusion |
|------------------------------|---|---|
| Population | Adultes atteints d'un déficit congénital sévère en alpha ₁ -antitrypsine (AAT), d'une maladie pulmonaire ou emphysème cliniquement manifeste associée à un génotype PiZZ, PiZ(nul), Pi(nul)(nul), PiSZ, ou tout autre allèle entraînant un déficit | Enfants atteints d'un déficit congénital en alpha ₁ -antitrypsine (AAT), adultes atteints d'un déficit congénital en alpha ₁ -antitrypsine (AAT) sans symptômes pulmonaires |
| Intervention(s) | Glassia ^{MC} | |
| Comparateur(s) | Placébo/meilleurs soins de soutien, thérapie d'augmentation avec un AAT plasmatique | |
| Résultats d'intérêt | Densité pulmonaire (tomodensitométrie thoracique), exacerbations, fonctions respiratoires, mortalité, innocuité, qualité de vie, efficience et impact budgétaire | |
| Temporalité | Aucune restriction de suivi | |
| Milieu d'intervention | Aucune restriction de site | |
| Période de recherche | À partir de 1987 | |
| Types de publications | Revue systématiques, méta-analyses, évaluation de technologies de la santé, études expérimentales et observationnelles, études qualitatives | Revue littéraire, série de cas (n < 5), éditoriaux, résumés, études de cas, études in vitro ou sur les animaux |
| Langue | Anglais et français | Autres qu'anglais et français |

ANNEXE C

Évaluation de la qualité méthodologique

L'évaluation de la qualité des études retenues pour l'évaluation de Glassia^{MC} a été effectuée de façon indépendante par deux examinateurs à l'aide de l'outil RoB 2.0.

Tableau C-1 Risque de biais évalué par l'outil RoB 2

| Études | Domaine 1 | Domaine 2 | Domaine 3 | Domaine 4 | Domaine 5 | Jugement global |
|---------------------------------|--------------------------|-----------|-----------|-----------|-----------|-----------------|
| [Sandhaus <i>et al.</i> , 2014] | Certaines préoccupations | Faible | Faible | Faible | Faible | Faible |

Notes : Domaine 1 : Biais provenant du processus de randomisation; Domaine 2 : Biais généré par des écarts par rapport aux interventions prévues; Domaine 3 : Biais généré par des données manquantes; Domaine 4 : Biais dans la mesure des paramètres d'intérêt; Domaine 5 : Biais dans la sélection du résultat rapporté.

ANNEXE D

Caractéristique du produit Glassia^{MC}

Tableau D-1 Caractéristiques du produit Glassia^{MC}

| Produit | Caractéristiques |
|--|--|
| Fabricant | Takeda Canada Inc. |
| Forme pharmaceutique | Solution pour injection |
| Teneurs | Flacon de 1000 mg / 50 ml (2 %) |
| Indication reconnue par Santé Canada | Pour le traitement d'augmentation et d'entretien de longue durée chez les adultes présentant un emphysème cliniquement manifeste attribuable à un déficit héréditaire sévère en inhibiteur de l'alpha1-protéinase (AAT), aussi appelé déficit en alpha1-antitrypsine (AAT). GLASSIA accroît les taux sériques d'IAI-P antigénique et fonctionnel (activité anti-élastase des neutrophiles). |
| Résumé des précisions sur l'indication | <ul style="list-style-type: none"> ▪ L'effet du sur les exacerbations pulmonaires et sur la progression de l'emphysème chez les patients présentant un déficit en AAT n'a pas été démontré dans le cadre d'ECR. ▪ Il n'y a pas de données cliniques étayant les effets à long terme du traitement. ▪ GLASSIA n'est pas indiqué pour le traitement de la maladie pulmonaire des patients chez qui un déficit sévère en AAT n'a pas été établi. |
| Voie d'administration | Perfusion intraveineuse, à une vitesse qui ne doit pas dépasser 0,2 ml/kg/min |
| Ingrédients non médicinaux | <ul style="list-style-type: none"> ▪ 0,02 M de phosphate de sodium ▪ chlorure de sodium à 0,7 % ▪ eau |
| Étapes de purification et inactivation des pathogènes | <ul style="list-style-type: none"> ▪ Sélection des donneurs ▪ Centres de collecte « American Red Cross » de plasma aux É.-U. ▪ Unités plasmatiques : tests sérologiques et acides nucléiques. ▪ Séparation des fractions II+III ▪ Précipitation au PEG ▪ Nanofiltration (15 nm) ▪ Traitement solvant/détergent (TNBT/Tween 80) ▪ Fractionnement éthanol froid à partir de la fraction IV-I du processus modifié de Cohn. |

| Produit | Caractéristiques |
|--|---|
| | <ul style="list-style-type: none"> ▪ 2 étapes de chromatographie, suivies d'une diafiltration et d'une ultrafiltration puis inactivation des pathogènes. Finalement, ultra + dia + filtration stérile. |
| Possibilité d'auto-administration | <p>Oui, ou par un proche-aidant, après une formation appropriée, et pourvu que l'utilisation du produit soit évaluée à intervalles réguliers. Les premières perfusions doivent être administrées sous la supervision d'un professionnel de la santé ayant de l'expérience dans ce traitement ou cette indication.</p> |
| Entreposage et stabilité | <ul style="list-style-type: none"> ▪ Conserver entre 2 et 8°C. ▪ Ne pas congeler. ▪ Température ambiante $\leq 25^{\circ}\text{C}$ pendant un maximum d'un mois. |

RÉFÉRENCES

Sandhaus RA, Stocks J, Rouhani FN, Brantly M, Strauss P. Biochemical efficacy and safety of a new, ready-to-use, liquid alpha-1-proteinase inhibitor, GLASSIA (alpha1-proteinase inhibitor (human), intravenous). *Copd: Journal of Chronic Obstructive Pulmonary Disease* 2014;11(1):17-25.

