FROM "DRUG" TO BLOOD PRODUCT

THE IMPACT ON PATIENTS

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Access for Alphas

- https://drive.google.com/file/d/1W3hrovaDRKXlOlnmNJgqnwKfVx1wgZ92/view

What is Alpha 1 Antitrypsin Deficiency

- Is an inherited genetic disorder
- Malfunctioning gene from each parent (ZZ)
- Resulting in low levels of A1AT (the major antiprotease in plasma} 0-57mg/dL or 0-11 mcmol/L
- Produced in liver, protective of lungs
- Deficiency Can cause
  - COPD : emphysema (damaged air sacs)
  - Liver disease, cirrhosis (liver scarring), cancer
  - Panniculitis (an uncommon skin condition)

Source: https://www.accessforalphas.ca/

https://my.clevelandclinic.org/health/diseases/21175-alpha-1-antitrypsin-deficiency

Diagnosis

How

- A1AT serum level
- Genetic testing

Recommendation

- Canadian Thoracic Society Guidelines (2012) for A1ATD target testing:
  - Test those with COPD diagnosed before 65 years or with smoking history of <20 pack years
  - Those with family history
  - Asthma patients with diagnostic uncertainty
- Individuals with unexplained liver disease

Source: https://cts-sct.ca/wp-content/uploads/2018/01/Alpha-1-GUIDELINE-2012.pdf

Source: https://www.accessforalphas.ca/

CTS Treatment Recommendations

- A1AT Augmentation therapy may be considered in nonsmoking or Ex smoking patients with COPD (FEV1 25%-80% predicted attributable to emphysema)

AND

- Documented A1AT level $\leq$ 11 mcmol/L (Normal 15.6-39.6 mcmol/L)

  < 57mg/dL (Normal 85-215 mg/dL)

- Source: https://cts-sct.ca/wp-content/uploads/2018/01/Alpha-1-GUIDELINE-2012.pdf

The ONLY Treatment

- Augmentation Therapy
  - Slows/ halts progression of emphysema (AATD)
  - Reduces exacerbations
  - Reduces hospitalizations
  - Prevents lung transplantation

Source: https://www.accessforalphas.ca/

My Story

- Undiagnosed 1999
- Infections MAC 2009
- Genetic testing

- CTS guidelines for treatment
  - serum level 24.4 mg/dL (0-57mg/dL)
  - FEV1 70% (Oct.2016),79% April 2017 (below 80%)

- 2017 Life Changed

Postal Code Lottery

- British Columbia
- Quebec – possibility
- Health Benefits- Coughlin, Manulife
  - Cost of treatment
- Angela Diano ED Alpha 1 Canada

Impact - THEN

- Very different approach as a DRUG
- Respirologist prescription- multiple insurance forms
- Prolastin Direct
- Prescription to Pharmacy
- Innomar - Prolastin Scheduling
- Delivery to Innomar clinic (private)
- Administration in clinic or Nurse to patient's home
- Product didn't touch patients' hands
- Only Product available in Canada
- May 2017 Grifols product to July 2024

How we got here

2017

ALPHA-1 CANADA first asked the nation's blood operator and their provincial/territorial (P/Ts) health ministry partners to consider an augmentation therapy category on the CBS national formulary in 2017. The P/Ts would not permit a review of the only augmentation therapy product in Canada, Prolastin-C (Grifols), for the purposes of establishing an augmentation therapy category due to the ambiguous CADTH criteria not permitting category reviews for products already being carried in the health system.

2019-2020

ALPHA-1 CANADA ENGAGED WITH CSL Behring Canada in hopes of partnering in a CADTH Plasma Protein Product Review between **2019-2020 IF** the provincial/territorial (P/Ts) health ministry partners would authorize it. P/Ts were still not interested in supporting an augmentation therapy category review.

2017-2020

ALPHA-1 CANADA LOBBIED provincial and territorial governments for 3+ years (2017-2020) to amend the CADTH criteria to permit a Plasma Protein Product Review; however, governments would only consider a review for products not in the health system.

2017-2022

ENGAGED IN FIVE-YEARS (2017-2022) of cross-country meetings with Provincial and Territorial Health Ministers and Deputy Ministers, Provincial Formulary Managers, and the Provincial Territorial Blood Liaison Committee (PTBLC)

2021-2022

PRESENTED AT BIANNUAL MEETINGS from 2017-2022 to the Canadian Blood Services (CBS) Board of Directors and CBS Executive.

2021

ALPHA-1 ANTITRYPSIN DEFICIENCY (AATD) is a rare disease with limited data, especially Canadian data, due to the slow progression of the disease and the small number of individuals impacted by this condition. Throughout 2021 Alpha-1 Canada used a mixed method approach to gathering patient and caregiver perspectives and experiences with alpha-1, providing as much comprehensive data as possible to submit to the Canadian Agency for Drugs and Technologies in Health (CADTH) for a Plasma Protein Product Review. Focus groups. Family interviews. Patient and caregiver surveys. Physician questionnaires.

FEBRUARY 2021 CBS NOTIFIED ALPHA-1 CANADA that P/Ts would allow a CADTH Plasma Protein Product Review for CSL Behring's Zemaira.

SUBMITTED TO CANADIAN AGENCY for Drugs and Technologies in Health (CADTH) to support the CSL Behring Zemaira IPPP Review in **October 2021**

WE ESTABLISHED A MICROSITE SPECIFICALLY FOR AN ACCESS FOR ALPHAS campaign, embedding a digital advocacy tool, allowing supporters to connect with politicians at the federal, provincial/territorial, and municipal level, through prepared letters that go directly to supporters' elected officials. Masses of letters sent from across Canada **2021-2022**

2022

RECEIVED A POSITIVE RECOMMENDATION from CADTH for Zemaira (CSL Behring) in Spring 2022.

APRIL 2022 Canadian Blood Services commenced their own 5-month review and budget impact assessment to determine if/how augmentation therapy could be established as a category within the Plasma Protein & Related Products Formulary.

THOUGH THERE ARE SPECIAL ACCESS PROGRAMS WITH RAMQ in Quebec to assist alpha-1 patients in QC to access augmentation therapy, Alpha-1 Canada believes that access should be transferred to Héma-Québec, because it would provide clarity and hopefully further accessible options for Alpha-1 patients to access plasma derived therapies as they are reviewed and approved as part of Héma-Québec's public formulary. As a blood product, Alpha-1 Canada believes this request is also aligned with the intent of the Act Respecting Héma-Québec and the Biovigilance Committee, that charges Héma-Québec with the responsibility to ensure sufficient supply of blood, blood products and blood components for the Québec population. For that reason, Alpha-1 Canada submitted to INESSS for Zemaira (CSL Behring), Prolastin-C Liquid (Grifols) & Glassia (Takeda) in **Summer 2022** to be considered for Héma-Québec's plasma formulary.

2023-2024

ALPHA-1 CANADA ESTIMATES THAT APPROXIMATELY 400-500 ALPHA-1 PATIENTS would be receiving life-prolonging treatment in 2023-2024, by gaining national access through Canadian Blood Services, depending on what current private and public payer providers opt to do with their reimbursement programs.

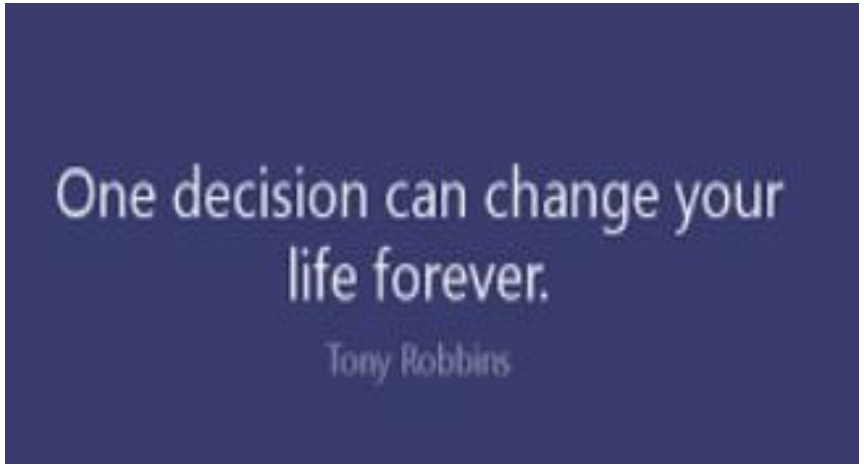
TIMELINE 2017 - 2024 | ACCESS FOR ALPHAS JOURNEY

ALPHA-1 CANADA

Alpha-1 Canada is a non-profit patient advocacy organization, providing education to patients and the healthcare community to increase awareness and testing for this genetic disease. Alpha-1 Canada has been appealing to Canadian Blood Services and the Provincial Territorial Blood Liaison Committee since 2017 to include treatment for alpha-1 patients on the national blood operator's plasma products formulary to ensure all Canadian alpha-1 patients have equal access to a plasma-derived replacement therapy.

How We got Here

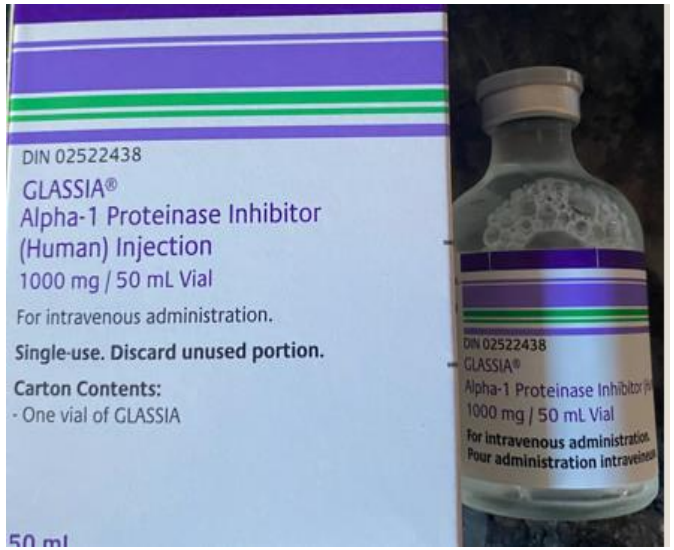
- Advocacy of Angela Diano on behalf of Patients 7 years
- Developing relationships
- Advocated get a CADTH plasma protein product review
- Positive CADTH recommendation Spring 2022
- CBS review and budget impact
- RFP for product
- All had to sign off
- Glassia (Takeda) CBS Formulary
- CBS leadership- Stakeholder engagement



CBS Stakeholder Transition

- CBS
- Takeda
- A1Canada-ED
- Transfusion Medicine Representatives –ON, BC
- Respirologists
- CTS
- Patient

Now



- Physician forms – CBS, Patient Support Program
- CBS review process if denied

- Patient Support Program Coordination
  - Clinic-Home-self infusion
  - Innomar scheduling
  - Transfusion Medicine/ Blood bank
  - Patient

- Patient Pick up
  - Transfusion Medicine/ Blood bank
  - Store Product
  - Transport to Clinic
  - Self-infusion

Differences

- Alberta delivery trial
  - Bayshore Pharmacy
- British Columbia
  - Working through process
- Quebec - Hema-Quebec
  - Submission for plasma formulary

HÉMA-QUÉBEC

Challenges

Respirologists

- Knowledge Gap
- Forms completion
- Workload
- No Incentive to transition?

Request for Patient Designated Plasma Protein and Related Products

Canadian Blood Services

■ **Glassia (alpha-1 proteinase inhibitor)**

Glassia may be requested for adult patients that meet ALL of the following criteria:**

- ■ Respirologist has confirmed the diagnosis of severe alpha-1 proteinase inhibitor (A1-PI) deficiency and clinical evidence of emphysema and indicated that patient would benefit from treatment with A1-PI product
- ☐ A1-PI deficiency, defined as serum A1-PI levels <11 µmol/L or < 57 mg/dL before start of the treatment
- ■ Clinical evidence of obstruction (FEV1 <80%)
- ■ Nonsmoker for at least 6 months
- ■ Has not received a lung transplant

Supporting Information (# required values)

Baseline serum A1-PI level#	24.4	☐ µmol/L ■ mg/dL
FEV1 (%)#	70%	

If baseline serum A1-PI level is unavailable, please clarify below:

- ■ Already on treatment with A1-PI product and no record of baseline level
- ☐ Other (explain):

A1-P1 baseline is from Oct.2016 prior to treatment

☐ **Other Product**:**

**If patient does not meet listing criteria or product is identified as "Other", an exceptional access review will be required. Please note that additional information may be requested, and the timeline for review may increase.

Current Therapy or ■ N/A

Product Name	Dose	Route of Administration	Frequency of Administration	Indication (e.g., prophylaxis, on demand)
Prolastin-C	4000mg	IV	q 1 week	maintenance

New Requested Therapy or ☐ Same as Current Therapy

Request for Patient Designated Plasma Protein and Related Products

Section IV: Total Contract Quantities in Vials
(refer to order form for product and available sizes)

Contracts will be created up to a maximum of **12 months**, A renewal request will be required every 12 months

Vial Size	**Total Contract Quantity**	**Pick Up Quantity**	**Frequency of Pick Up** (e.g., every 3 months)	**Duration of Contract** (max 12 months)
1000mg/50 ml	208	52	q 3 months	12 months

Date of next product order (please comment if less than 1 week):	Comments (please include when next dose is due for STAT requests):
Expiry date of approved contract (optional to fill out for records following CBS notification):	

Order Form For Plasma Protein and Related Products Requiring Contracts

Site: >The Ottawa Hospital -General campus

Hospital/Customer: The Ottawa Hospital Phone /Fax: 6137396807 Date: March 11/2024 Time: 1200

City/Town: Ottawa Requested By: Dr G Alvarez

Delivery Priority: Routine ■ ASAP □ *STAT □ [*STAT orders must be faxed and phoned]

Delivery Mode: Patient pick up Date Needed: Ship to Location: TOH-Civic Transfusion Medicine

CONTRACT NUMBER: To be provided by CBS

- **Initial requests** ensure the appropriate request form for the product specified is provided
- **Urgent initial requests** for **Panhematin** do not require a contract
- **ONLY one contract number per order form can be accepted**

CBS Code	Product/Manufacturer	Vial Size	Number of Vials	To Be Filled (For CBS Use Only)
Monoclonal Antibody				
1000107699	Hemlibra®, emicizumab, Roche	30 mg/1mL		
1000107700	Hemlibra®, emicizumab, Roche	60 mg/0.4 mL		
1000107701	Hemlibra®, emicizumab, Roche	105 mg/0.7mL		
1000107702	Hemlibra®, emicizumab, Roche	150 mg/1mL		
Hemin for Injection				
1000107704	Panhematin®, Recordati Rare Diseases Canada	268 mg		
Alpha-1 Proteinase Inhibitor				
1000109400	Glassia, Takeda	1000 mg/ 50 mL	4	
Other Product				

Challenges

- Transfusion Medicine
  - Life and Death situations-code bleed
  - Critical clinical area
  - Workload
  - No patient access
  - Space
  - Limited TMs
  - Lab Information System change

Potential Solutions

- Consolidate Pick Up to most appropriate Transfusion Medicine Department/ BB
- Consider potential patient volume (Windsor >5/year, Burlington 5-6/year)
- "Distribution Modernization"

Challenges

- Patients
  - BC, private payor –no incentive
  - Resistance to change
  - Storage: Weight based dosing
  - Pick up of 3-month supply
  - Fridge vs $100,000+ per year, co pay?
  - Challenge to get to hospital patient condition, parking, walking, O2 etc
  - Location of TM

The Numbers

- 2021
  - 313 patients receiving augmentation therapy
  - 107 public payer
  - 230+prescribed but had no access
  - Dying while waiting

- 2024
  - All have access
  - Alpha 1 Canada estimates 400-500 patients would be receiving life prolonging treatment
  - 3-5 Alphas who relocated to BC/year can return home

Source: https://www.accessforalphas.ca/

Edmonton, first home delivery

What is the Impact

- Augmentation is the **ONLY** Treatment for the genetic disease
- Early diagnosis and treatment improves longevity
- Reduces risk of severe lung disease
- Reduces hospitalization
- Allows Alpha 1 people to remain in their communities
- Supports family structure
- It allows us to live a full life


感謝你
Danke
Gracias
Grazie
Thanks!
THANK
YOU!