ACCESS FOR ALPHAS JOURNEY

ALPHA-1 CANADA is a national non-profit organization committed to advocating on behalf of Canadians affected by Alpha-1 Antitrypsin Deficiency (AATD). Alpha-1 Canada believes all patients that require augmentation therapy should have equitable access to treatment. The organization's position is aligned with clinical practice guidelines in Canada and around the world, which have recognized that intravenous augmentation therapy can slow or even halt the accelerated progression of emphysema due to AATD, preventing exacerbations, hospitalizations, and lung transplantation.

FACTS

Alpha-1 Antitrypsin Deficiency is a genetic disorder, with the prevalence considered to be **1 IN 5,000 PEOPLE IN CANADA.**

Alpha-1, a rare hereditary condition characterized by low circulating levels of alpha-1 antitrypsin (AAT) protein in the blood, leads to increased risk of lung and liver disease.

There are currently **1,327** patient and caregiver members and **300** health care providers enrolled with Alpha-1 Canada.

ALPHA-1 CANADA OBTAINED 12 FORMAL ENDORSEMENTS FOR THE LISTING OF AUGMENTATION THERAPY WITH CANADIAN BLOOD SERVICES FROM:

CAGC - ACCG

Société canadienne de thoracologie / Canadian Thoracic Society

BREATHE the lung association

Alpha-1 Canadian Registry

AlphaNet Canada

CVAA ACAV Canadian Vascular Access Association / Association Canadienne d'Accès Vasculaire

ALPHA-1 FOUNDATION

Réseau canadien pour les soins respiratoires / Canadian Network for Respiratory Care

airways group

Canadian Society of Respiratory Therapists / Société canadienne des thérapeutes respiratoires

PPTA Plasma Protein Therapeutics Association

NRBDO RAVTSR Network of Rare Blood Disorder Organizations / Réseau des Associations Vouées aux Troubles Sanguins Rares

In 2021 there were **313** Canadian patients receiving augmentation therapy, only **107** of them through a public payer; however, **230+** additional Canadians had been prescribed but they have no public nor private coverage to access treatment.

From a medical, biological and biopharmaceutical perspective there is no rational difference between augmentation therapy and the other plasma-derived products that Canadian Blood Services distributes. There is no technical reason that augmentation therapy should not be classified in the exact, same manner as all the other listed products by Canadian Blood Services.

We raised awareness about the lack of access to a blood product by signaling public opinion to decision-makers, through an online petition, which garnered **3,000+ SIGNATURES.**

It takes **900 PLASMA DONATIONS** per year to help one patient with AATD.

- Developed **10 DOCUMENTARY-STYLE PATIENT AND PHYSICIAN FILMS,** featured on the ACCESS FOR ALPHAS microsite and streamed through social media platforms.
- Developed an ACCESS FOR ALPHAS social media campaign, which included original content that earned media featuring patients' stories, while also informing the patient community of HTA review milestones.
- Press releases specific to local patient stories resulted in earned media coverage with: Toronto Star, Calgary Herald, Edmonton Journal, St. Catharines Standard, SaltWire Network, Niagara This Week, Medium, Global News, CBC/Radio-Canada.

TIMELINE 2017 - 2024 | ACCESS FOR ALPHAS JOURNEY

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.

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)

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

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.

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**

2021-2022

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.