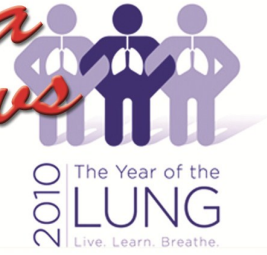




# Alpha-1 Canada Community News



October 2010

What's New at  
[www.alpha1canada.ca](http://www.alpha1canada.ca)

- Ontario withdraws funding for augmentation therapy, join the fight to reinstate

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## Canadian Organization for Rare Disorders' Day of Action on the Hill

by Vanessa McLaughlin

On September 30th, 2010, Canadian patients, family members and supporters of people with rare disorders joined together with one voice to advocate for a regulatory framework for rare disorders in Canada that would include a definition of a rare disorder.

I was happy to be among them and thankful that Ken Bee, an Alpha-1 patient who is very active in Alpha-1 Canada advocacy issues, was able to join me.

According to the Canadian Organization for Rare Disorders' (CORD), Canada does limited research on rare diseases, contributes little to the development of treatments of rare diseases, and offers poor access to treatment for Canadians with rare diseases. Canada is one of the only developed countries without a policy for rare disorders.

The development and implementation of a regulatory framework for rare disorders is needed in Canada in order to provide incentives for the development of new drugs, guidance in conducting clinical trials and pathways to approval. Until this happens, Canadian patients suffering from rare diseases will continue to face poor access to diagnosis, care and treatment in Canada.

To date, the Government has recognized the need to move forward on this criti-



Person A, Brian Masse, MP, Person B, person C and Vanessa McLaughlin

cal issue, but there has been no concrete action.

To maximize impact with decision makers in a single day, 14 delegate teams met with 51 members of Parliament. MPs were informed about the challenges of rare disorders, asked to advocate for a federal regulatory framework and urged to write a letter to the Prime Minister and Health Minister calling for a regulatory framework for orphan drugs and rare disorders.

I had the opportunity to meet with NDP MP Don Davies, Vancouver Kingsway, BC and NDP MP Brian Masse, Windsor West, ON (top right photo). Both were receptive, engaging and committed themselves to writing a letter to the Health Minister.

In order to maintain the momentum, write a letter to your MP or request a meeting asking them to commit to writing a letter

to the Health Minister. A sample letter can be found here. Be sure to include your personal story in the letter. If you are unsure of who your member of parliament is or where to write to, you can find him or her using your postal code at the following web site: <http://www2.parl.gc.ca/parlinfo/compilations/houseofcommons/memberbypost-alcode.aspx?menu=hoc>.

If you don't engage with people that impact your issue, someone else will with a competing concern and interest. Please ensure this issue is a top priority as you do have the power to affect change. Most importantly, your life and the lives of others are at stake.

Health Canada is holding technical consultations on a regulatory framework for orphan drugs, scheduled for October 27.

## Inhaled alpha-1 antitrypsin treatment is a step closer in Canada

by Jim Mundy

In an e-mail in August we shared with you the news about Baxter International Inc. acquiring exclusive rights to distribute Kamada's Glassia™ alpha-1 augmentation therapy in Canada.

Baxter International Inc. and Kamada Ltd. announced then an agreement whereby Baxter acquired the exclusive right to distribute and ultimately manufacture Kamada's intravenous liquid AAT product, Glassia™ in the United States and distribute it in Canada, Australia and New Zealand.

Kamada is headquartered in Israel. There is still no word on when Glassia™ will be available in Canada. In August we told you that the distribution rights and the licensing agreement did not include the inhaled version of Kamada's AAT product,

currently in phase 2-3 clinical trials in Europe, but that under the terms of the agreements, both sides would examine additional cooperation opportunities for the inhaled product.



We recently learned that Baxter president Larry Guiheen visited Kamada's offices and factory in Israel in October, meeting with senior executives, including Kamada CEO David Tsur.

An anonymous market source said, "Guiheen's visit says it all: Baxter is trying to accelerate as much as possible the completion of the next stage of the deal,

regarding inhalation, and the sooner the better. Kamada, for its part, is trying obviously to maximize the payment for the next stage."

No one is making any commitments, however, circumstances seem to be lining up to make the possibility of inhaled AAT augmentation therapy being available in Canada more likely.

Inhaled AAT is seen as the 'next generation' of treatment for alpha-1 antitrypsin deficiency, bronchiectasis and cystic fibrosis. Kamada's product utilizes a special nebulizer and has been designated orphan drug status in both Europe and the U.S. Canada, as you know, still does not have an orphan drug policy, although that should not affect the introduction of inhaled AAT augmentation therapy in Canada.

## Fundraising for rare disorders

by Vanessa McLaughlin

When children and adults are being diagnosed with a rare disease, such as Alpha-1, it is a real challenge to get information and support.

A further challenge is to get researchers interested as it affects only a tiny percentage of the Canadian population. Until we have a more significant commitment at the provincial and federal level, the emotional and financial burden of funding and driving information, support and research on rare diseases will fall upon patients and their families.

Through events such as golf tournaments, sponsored

walks and runs, spaghetti suppers and proceeds of garage sales, communities are helping families fund treatment and research.

Canadians are generous, donating both time and money to charity. Fundraising events in your community can also raise awareness about Alpha-1 and rare diseases.

If you have or are currently involved in a fundraising event to raise funds for Alpha-1, please tell us your story. If you are interested in volunteering your time and ideas, help getting started is just a phone call away. We all have to do our

part. It's not as difficult as you may think. You will be amazed how local events can significantly contribute in so many ways.



### Tip of the month

One small step can pay big health dividends over time.

Strolling just 30 minutes a week benefits your health.

### What is a rare disorder?

A rare disorder is a disease or disorder which has a very low prevalence rate.

Rare disorder is defined in most countries as affecting fewer than 1 in 2,000 persons.

Canada does not have a definition of rare disease.

## Your Liver

The liver performs over 500 different functions. In fact it is so important that we cannot live without our liver.

Every minute your liver filters more than a liter of blood. All blood runs through your liver and it filters out different toxins, including chemicals, alcohol, recreational drugs and pesticides.

Signs of a liver problem include headaches, indigestion, constipation, stomach cramps and weight gain. Some people also may notice that they can't eat spicy foods or greasy foods without feeling sick.

You can keep your liver healthy by cutting back on alcohol and processed or fried foods. You can also stop overeating, which may put too much pressure on your liver. Your liver can regenerate (re-build) itself. Even if only 25% of it is still healthy your liver can regenerate itself into a full liver again.

## Potential new therapies to treat ATT deficiency

Talecris, the manufacturer of Prolastin©-C, awarded two 50,000 euros fellowships to young researchers who aim to enhance the understanding and treatment of alpha-1 antitrypsin deficiency.

The recipients of the 2010 European alpha-1 antitrypsin Laurell's Training Awards (eALTA) are Sandra Pelz, postgraduate research fellow at the Martin-Luther University in Halle-Wittenberg, Germany and Dr Adriana Ordonez, post-doctoral research fellow at the Cambridge Medical Research Wellcome Trust.

### ATT and Influenza

Omni Bio Pharmaceutical has entered a two-year research agreement with the University of Colorado Denver (UCD), and in collaboration with researchers at National Jewish Health, to evaluate the biological activity of Alpha-1 antitrypsin (AAT) and its effects on influenza.

"In preclinical studies, AAT has demonstrated the potential inhibition of several medical disorders, including influenza. Given its broad therapeutic potential, AAT may be able to inhibit a variety of influenza infections, including seasonal,

The award is named in honour of Dr Carl-Bertil Laurell, who first described Alpha-1 in 1963.

"Research initiatives such as eALTA not only increases our understanding of the disease, but they also help stimulate interest and commitment of early career scientists and clinicians who represent the future of research and new treatments for the Alpha-1 community," said Claus Vogelmeier, Professor for Internal and Respiratory Medicine and Chair of the independent eALTA Review Team.

Pelz's research project will use a mouse model to investigate the potential use of stem cell-derived liver cells, known as hepatocytes, to correct the genetic defect that gives rise to liver disease associated with ATT deficiency.

Ordonez's project will study the mechanism by which small molecule inhibitors can block the Alpha-1 protein from abnormally folding and accumulating in the endoplasmic reticulum of hepatocytes.

Source: PR Newswire

bird and swine related influenzas.

While vaccines on the market and in development are primarily for prophylactic use in healthy individuals to prevent influenza infection, AAT may effectively inhibit influenza symptoms in infected patients who require immediate treatment," said Dr. Leland Shapiro, principal investigator of the study at UCD.

Positive results from this study would allow Omni Bio Pharmaceutical to file an investigational new drug application to the U.S. Food

and Drug Administration and initiate clinical trials of AAT patients who have contracted severe forms of influenza, such as H1N1 or bird flu.

Source: Marketwire via Comtex



## Rare Diseases Emerging Teams: Translating Basic Biology to Enhanced Patient Care

The Canadian Institutes of Health Research (CIHR) Institutes of Genetics and Nutrition, Metabolism and Diabetes - in partnership with the CIHR Institutes of Cancer Research, Circulatory and Respiratory Health, Gender and Health, Health Services and Policy Re-

search, Musculoskeletal Health and Arthritis, with The Ataxia of Charlevoix-Saguenay Foundation, The Canadian Cystic Fibrosis Foundation, The Canadian Organization for Rare Disorders, The Foundation Fighting Blindness - Canada, and The Kidney

Foundation of Canada - are pleased to announce the "Rare Diseases Emerging Teams: Translating Basic Biology to Enhanced Patient Care" funding opportunity in support of the creation of collaborative, *continued on page 4*

## Rare Diseases Emerging Teams: Translating Basic Biology to Enhanced Patient Care

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novel, interdisciplinary research teams addressing important research questions focused on rare genetic diseases.

To date, more than \$14 million over five years has been secured to fund collaborative, novel, interdisciplinary teams focused on either (i) the basic biology, and/or undertaking clinical research, and/or conducting research on the populations affected by the rare disease or (ii) the health services, policy, economics and/or ethical, legal or social issues related to rare diseases.

For the purposes of this opportunity a rare disease is defined as one that affects 1 in 2,000 or fewer people, and will include:

Single gene or monogenic disorders

Diseases that are not monogenic, but in which the genetic contribution is complex

Uncommon familial forms of genetically complex disorders, such as Alzheimer's disease, hypertension, diabetes, coronary artery disease, musculoskeletal disorders, and rare cancers to cite a few of many examples

The total amount currently available for this funding opportunity is \$14.5 million over five years.

Up to \$11.25 million has been allocated in support of teams investigating the basic biology, and/or undertaking clinical research, and/or conducting research on the populations affected by the rare disease. The maximum grant to a team in this area will be up to \$500,000 per year for five years.

Up to \$3.25 million has

been allocated in support of teams focused on health services, policy, economics and/or ethical, legal or social issues related to rare diseases. The maximum grant to a team in this area will be up to \$300,000 per year for five years.

THIS FUNDING OPPORTUNITY IS SCHEDULED TO BE LAUNCHED MID-OCTOBER 2010 AND FULL PROGRAM DETAILS WILL BE AVAILABLE THEN. For questions about this strategic initiative and research objectives, please contact:

Stephanie Robertson

Assistant Director, Institute of Genetics  
Canadian Institutes of Health Research  
Telephone: 613-954-0533  
Email: stephanie.robertson@cihr-irsc.gc.ca

### Liver Disease

The lack of alpha-1 antitrypsin is the most common cause of genetic liver disease in children.

In adults with alpha-1 antitrypsin deficiency male gender and obesity may predispose to advanced liver disease. Emphysema is the cause of death in most patients. Five percent of deaths are attributed to liver disease.

## World COPD Day

World COPD Day is an annual event organized by the Global Initiative for Chronic Obstructive Lung Disease (GOLD) to improve awareness and care of chronic obstructive pulmonary disease (COPD) around the world.

World COPD Day (WCD) 2010 will take place on November 17 around the theme "2010 - The Year of the Lung: Measure your lung health - Ask your doctor about a simple breathing

test called spirometry."

As this year's theme indicates, World COPD Day 2010 is part of the Year of the Lung, a global effort throughout 2010 to raise public and governmental awareness of the burden of respiratory diseases.

As the initiative's organizers put it, "Hundreds of millions of people around the world suffer every day from treatable and preventable respiratory diseases. Respiratory diseases rank second (after cardiovascular diseases) in terms of mortality, incidence, prevalence and costs.

COPD, pneumonia, tuberculosis (TB) and lung cancer are the main respiratory

causes of death worldwide."

WCD organizers around the world are, planning ambitious events to help raise COPD awareness and improve diagnosis and care.

Here are just a few examples of planned activities that pick up on this emphasis on spirometry.

In Ireland, the Mayo General Hospital will offer spirometry testing and advice about COPD. Members of the Mayo COPD Support Group will be present to speak with those tested; they will provide positive messages about living with COPD and encourage those

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**November 17 2010**



Follow us on Twitter.

We've learned that Social Media people care about their fellow men, women, children, animals and the environment. They exist in a system that is supported by the camaraderie and sharing of others.

It's no small wonder then that non-profits abound on Twitter. With the free and easy-to-setup interface, a potential to reach a vast audience, the ability to engage with people in conversation directly, and the possibility to garner an audience more open to causes than advertisements, it seems a no-brainer for any organization seeking philanthropy and recognition for their mission.

Please follow us, tweet to us, tweet about us and retweet our tweets. We promise to do likewise. Together we can use these media opportunities to raise awareness and support for people affected by Alpha-1. Just think of the possibilities and how YOU can make them happen!

## World COPD Day

*continued from page 4*

with COPD to join the support group. In addition, the support group will host tables in eight locations around Ireland to sell GOLD Awareness ribbons and hand out literature about COPD and the support group.

In India, Rohilkhand Medical College will offer a free spirometry camp. Faculty at the college will reach health professionals with a half-day continuing medical education event on COPD.

In the USA, Cabell Huntington Hospital will offer spirometry testing and information about COPD. The event will feature a pulmonologist, pharmacist, respiratory therapist, home

-care specialist, and social workers. Visitors will be able to register for a free spacer give-away and obtain free informational materials.

In Canada, the Lung Association of Saskatchewan – which will be 100 years old on February 17, 2011 – will be hosting the Coast-2-Coast Challenge, a virtual trek across Canada, to raise awareness about COPD and to encourage people to speak to their health care provider for more information about

testing for COPD.

Anyone can join this free event. Any physical activity, such as walking, jogging, swimming or biking, can count towards the total distance tabulated on a virtual map that extends from coast to coast along the TransCanada Trail.

For more information or to register distances covered, please contact Bernie by phone (1-888-566-5864) or by email at [bernie.bolley@sk.lung.ca](mailto:bernie.bolley@sk.lung.ca).



## INTERESTING RESEARCH

*The following are summaries (abstracts) of recent studies of Alpha-1 and COPD. Please note that Dr. Chapman (pictured below), who is the chair of Alpha-1 Canada's Medical Advisory Board, is the co-author of the study in the first abstract.*

*Because of copyright law we can only provide abstracts, if you want to read more check and see if your local library has these journals on their shelves.*



**Pharmacokinetic comparability of Prolastin®-C to Prolastin® in alpha1-antitrypsin deficiency: a randomized study.**

Stocks JM, Brantly ML, Wang-Smith L, Campos

MA, Chapman KR, Kueppers F, Sandhaus RA, Strange C, Turino G.

BMC Clinical Pharmacology. 2010 Sep 30;10(1):13. [Epub ahead of print]

### Abstract

**BACKGROUND:** Alpha1-antitrypsin (AAT) deficiency is characterized by low blood levels of alpha1-proteinase inhibitor (alpha1-PI) and may lead to emphysema. Alpha1-PI protects pulmonary tissue from damage caused by the action of proteolytic enzymes. Augmentation therapy with Prolastin® (Alpha1-Proteinase Inhibitor [Human]) to increase the levels of alpha1-PI has been used to treat individuals with AAT deficiency for over 20 years. Modifications to the Prolastin manufacturing process, incor-

porating additional purification and pathogen-reduction steps, have led to the development of an alpha1-PI product, designated Prolastin(R)-C (Alpha1-Proteinase Inhibitor [Human]). The pharmacokinetic comparability of Prolastin-C to Prolastin was assessed in subjects with AAT deficiency.

**METHODS:** In total, 24 subjects were randomized to receive 60 mg/kg of functionally active Prolastin-C or Prolastin by weekly intravenous infusion for 8 weeks before crossover to the alternate treatment for another 8 weeks. Pharmacokinetic plasma samples were drawn over 7 days following last dose in the first treatment period and over 10 days following the

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## INTERESTING RESEARCH

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last dose in the second period. The primary end point for pharmacokinetic comparability was area under the plasma concentration versus time curve over 7 days post dose (AUC<sub>0-7</sub> days) of alpha1-proteinase inhibitor determined by potency (functional activity) assay. The crossover phase was followed by an 8-week open-label treatment phase with Prolastin-C only.

**RESULTS:** Mean AUC<sub>0-7</sub> days was 155.9 versus 152.4 mg\*h/mL for Prolastin-C and Prolastin, respectively. The geometric least squares mean ratio of AUC<sub>0-7</sub> days for Prolastin-C versus Prolastin had a point estimate of 1.03 and a 90% confidence interval of 0.97-1.09, demonstrating pharmacokinetic equivalence between the 2 products. Adverse events were similar for both treatments and occurred at a rate of 0.117 and 0.078 per infusion for Prolastin-C (double-blind treatment phase only) and Prolastin, respectively ( $p = 0.744$ ). There were no treatment-emergent viral infections in any subject for human immunodeficiency virus, hepatitis B or C, or parvovirus B19 during the course of the study.

**CONCLUSION:** Prolastin-C demonstrated pharmacokinetic equivalence and a comparable safety profile to Prolastin. Trial Registration: ClinicalTrials.gov Identifier: NCT00295061.

**Falls in people with chronic obstructive pulmonary disease: An observational cohort study.**

Roig M, Eng JJ, Macintyre DL, Road JD, Fitzgerald JM, Burns J, Reid WD. Department of Physical Therapy, University of British Columbia, Vancouver, Canada; Muscle Biophysics Laboratory, University of British Columbia, Vancouver, Canada.

Respiratory Medicine 2010. Sep 22. [Epub ahead of print]

### Abstract

**STUDY OBJECTIVE:** To investigate incidence, risk factors and impact of falls on health related quality of life (HRQoL) in patients with chronic obstructive pulmonary disease (COPD).

**DESIGN:** Observational cohort study.

**METHODS:** Patients completed these questionnaires at baseline and at 6-months: Medical Outcomes Study Short Form 36 (SF-36), Chronic Respiratory Questionnaire (CRQ), Activities Balance Confidence (ABC) Scale and a form to record demographic data, medications, co-morbidities, oxygen use, acute exacerbations, fall history and assistive device use. Physical activity was measured with the Physical Activity Scale for the Elderly (PASE) only at baseline. Fall incidence was monitored through monthly fall diaries. Patients were categorized as non-fallers (0 falls) or fallers ( $\geq 1$  falls).

**RESULTS:** Data from 101 patients with a forced expiratory volume in 1 s of  $46.4 \pm 21.6\%$  predicted were analyzed. Thirty-two patients (31.7%) reported at least one fall during the 6-months. Fall incidence rate was 0.1 (95% CI: 0.06

-0.14) falls per person-month. Fallers tended to be older ( $p = 0.04$ ), female ( $p = 0.04$ ) and oxygen dependent ( $p = 0.02$ ), have a history of previous falls ( $p < 0.001$ ), more co-morbidities ( $p = 0.007$ ) and take more medications ( $p = 0.001$ ). Previous falls (OR = 7.36; 95% CI: 2.39-22.69) and diagnosis of coronary heart disease (OR = 7.07; 95% CI: 2.14-23.36) were the most important predictors of falls. The Dyspnea Domain of the CRQ declined significantly more ( $p = 0.02$ ) in the fallers group at 6-months.

**CONCLUSIONS:** Patients with COPD have a high susceptibility to falls, which is associated with a worsening of dyspnea perception as related to HRQoL. Fall prevention programs in COPD are recommended.

### Osteoporosis in COPD outpatients based on bone mineral density and vertebral fractures.

Graat-Verboom L, van den Borne BE, Smeenk FW, Spruit MA, Wouters EF. Department of Respiratory Medicine Catharina Hospital Eindhoven Eindhoven, the Netherlands.

Journal of Bone and Mineral Research. 2010 Sep 27. [Epub ahead of print]

### Abstract

One of the extra pulmonary effects of chronic obstructive pulmonary disease (COPD) is osteoporosis. Osteoporosis is characterized by a low bone mineral density (BMD) and micro architectural deterioration. Most studies in COPD

*continued on page 7*

## Liver Research

The Alpha-1 Foundation's Liver Report is now available on their website.

The report summarizes the first 10 years of Foundation-sponsored liver research and announces their new Liver Initiative, aimed at intensifying their research efforts on Alpha-1 liver disease.

Read it here : <http://www.alpha-1foundation.org/alphas/?c=Liver-Research-Update>

If you know of any research, articles or other publications that would be of interest to our readers please contact us.

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Visit us on the web  
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Our website is continuously updated with useful information for Alphas, their caregivers and healthcare providers, as well as news on promising research. Make a habit of checking our website regularly so you won't miss out on exciting updates and always read our monthly newsletter from top to bottom.

Help us spread awareness by sharing this newsletter with your family and friends.

If you would like to receive this newsletter by e-mail, please contact us at 1-888-669-4583 or [vanessa.mclaughlin@alpha1canada.ca](mailto:vanessa.mclaughlin@alpha1canada.ca)

This newsletter is designed to support, not replace, the relationship that exists between you and your physician. It is not the intention of this newsletter to provide specific medical advice but rather to provide the Canadian Alpha-1 Community with information to better understand their health and their diagnosed disorder.

Specific medical advice will not be provided and Alpha-1 Canada urges you to consult with a qualified physician for diagnosis and for answers to your personal questions.

**Alpha-1 Canada**  
**Making a difference in the lives of Alphas**

## INTERESTING RESEARCH

*continued from page 6*

patients use dual energy absorptiometry (DXA) scan only to determine osteoporosis, therefore micro architectural changes without a low BMD are missed. Aim of the current study was to determine the prevalence and correlates of osteoporosis in COPD patients based on DXA-scan, X-ray of the spine (X-spine) and the combination thereof. DXA-scan, X-spine, pulmonary function

testing, body composition, 6-minutes walking distance, medical history and medication use were assessed in 255 clinically stable COPD outpatients of a large teaching hospital in the Netherlands. Half of all patients had radiological evidence for osteoporosis. Combining the results of DXA-scans with X-spine augmented the proportion of COPD patients with osteoporosis compared to both methods separately. The prevalence of osteoporosis was not

significantly different after stratification for GOLD-stage. Most patients with osteoporosis did not receive pharmacological treatment. Age, body mass index (BMI) and parathormone (PTH) were significant independent correlates for osteoporosis. Chest physicians should be aware of the high prevalence of osteoporosis in COPD even in case of a low GOLD score. Especially in elder COPD patients with a low BMI and/or an increased PTH.

