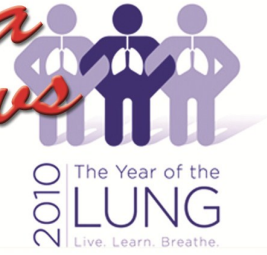




Alpha-1 Canada Community News



November 2010

What's New at
www.alpha1canada.ca

- **Baxter acquires exclusive right to distribute Kamada's Glassia™ alpha-1 augmentation therapy in Canada**
- **Drop-in Support Group meeting on Thursday December 2, 2010 with guest speaker Dr. Durhane Wong-Rieger**

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The development and implementation of a Canada-wide regulatory framework for rare disorders – Promises kept?

by Vanessa McLaughlin

On behalf of Alpha-1 Canada, I am pleased to invite you to our drop-in meeting on Thursday, December 2nd at 7 PM (Eastern Time).



Our guest speaker will be Durhane Wong-Rieger (pictured above), president and CEO of the Institute for Optimizing Health Outcomes. She is also president of the Canadian Organization for Rare Disorders (CORD) and head of Consumer Advocare Network, a national network to promote patient engagement in healthcare policy and advocacy.

Internationally, she serves as Chair-Elect of the Board of the International Alliance of Patient Organizations.

As president of the Canadian Hemophilia Society, Durhane advocated on be-

half of victims of tainted blood and was named to the Board of Directors of the Canadian Blood Services and the National Blood Safety Council.

Dr. Wong-Rieger has served on numerous health policy advisory committees and panels, including Project Coordinator for the Policy Dialogues for the Commission on the Future of Healthcare in Canada and consultant to the Ontario Premier's Advisory Body on Organ Donation.

Dr. Wong-Rieger will be discussing the development and implementation of a Canada-wide regulatory framework for rare disorders.

Such a framework is needed to provide incentives for the development of new drugs, guidance in conducting clinical trials and pathways to approval.

Canada is one of the only developed countries in the world without a standard definition of a rare disorder, let alone a regulatory framework for rare disorders or a national plan.

In fact, Canada does limited research on rare diseases, contributes little to the development of rare diseases, and offers poor access to treatments for Canadians with rare diseases. This is shocking when it is estimated that one in 12 Canadians has a rare condition.

After years of lobbying by patient groups, Health Canada finally seems prepared to change the way drugs are approved to accommodate rare diseases and the orphan drugs needed to treat them.

In late October, Health Canada held technical consultations on a regulatory framework for orphan drugs. Draft regulations were to be drafted after the consultation period.

Dr. Wong-Rieger will discuss the changes that will eventually be made as part of wider modernizations to Health Canada and whether the Government is ready to move forward on a regulatory framework for rare disorders.

You can also do something to advocate for such a framework by writing a letter to your MP asking them to commit to writing a letter to the Health Minister. A sample letter can be found on our website [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-alcodes.aspx?menu=hoc>.

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Prolastin®-C roll-out complete in Canada

by Jim Mundy

In our March 2010 newsletter we told you that Talecris Biotherapeutics announced that it had received approval from Health Canada for Prolastin®-C, a more purified and concentrated version of Prolastin®. Prolastin-C is produced using advances in manufacturing technology. A similar approval was granted in the U.S. by the Food and Drug Administration for Prolastin-C on October 17, 2009.

In a recent news release, Talecris announced that the roll-out of Prolastin®-C in Canada is complete and all patients receiving Prolastin AAT augmentation therapy are now receiving Prolastin-C.

Prolastin-C is the only AAT augmentation therapy ap-

proved for use in Canada.

For those Alphas receiving augmentation therapy there are a few important differences between Prolastin and Prolastin-C that your pharmacist will have told you about.



First, Prolastin®-C is prepared for infusion by mixing it with sterile water. For Prolastin®-C each 1,000 milligram vial is reconstituted using 20 millilitres of sterile water, not the 40 millilitres that was used for Prolastin.

This explains the second difference — shorter infusion time, since PROLASTIN

-C delivers 2 times the active protein (AAT) per milliliter. This can reduce infusion time by half.

The third difference between the two Prolastins is higher purity. According to Talecris, each dose of Prolastin-C delivers approximately 90% pure alpha-1 protein allowing it to be more concentrated.

If you are currently receiving Prolastin-C, we would love to hear from you. Although receiving reimbursement for the cost of this therapy is difficult in most parts of Canada there are people currently considering this therapy that would benefit from hearing about the experience of people already on therapy.

Canada ranks low internationally on pharmaceutical reimbursement

by Vanessa McLaughlin

On Wednesday, November 3rd, Rx & D (Canada's Research-Based Pharmaceutical Companies), held a live interactive video webcast to launch the findings of their 4th annual Report on Access to Medicines (IRAM) 2009-10. This report compares pharmaceutical reimbursement in Canada with other developed nations that have the ability to pay for health services, including drugs. The report also measures their willingness to pay for new, innovative pharmaceuticals.

Despite ranking in the top 10 in both GDP per capita and percent of GDP spent on health, Canada ranks 23rd out of 29 OECD countries in terms of public plan cover-

age of new medicines.

"These findings will surprise Canadians. The IRAM Report reveals that less affluent countries than Canada are finding ways to provide greater access to innovative medicines through their public drug plans," said Russell Williams, President of Rx & D. "Our offer is to work with governments and other health care providers to promote innovation as a tool to improve patient health and build a more sustainable health care system."

Of interest to Alpha-1 patients is that treatment and drugs for rare diseases are not being reimbursed to the same extent we see in other countries. Also, as many of you are all too aware of, where you live in Canada or

the "geography lottery" also influences access to treatment. This is beyond frustrating as access is about providing all Canadians in need with proper health care. According to the speaker, the next phase of research is to find out why there is such a discrepancy between Canada and other countries, to make innovative medicines available for rare disorders as well as make every disease a priority. However, this highlights -once again- the need for a national framework on rare disorders in Canada.

To read the full report on access to medicines across Canada, go to www.patientscomefirst.ca.

Tip of the month

Caring for a loved one?

Support, stress-relief and strength starts with self-care.

Read more about Self-care stress relief for caregivers : [http://www.morethanmedication.ca/en/article/index/Caregiver Stress](http://www.morethanmedication.ca/en/article/index/Caregiver%20Stress)

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.

Donations

Find out how you can give gifts that don't need to be wrapped and provide information, education and support to people who could really use your help. Contact Alpha-1 Canada at 1-888-669-4583 or vanessa.mclaughlin@alpha1canada.ca for more information.

We would like to take this opportunity to send out a hearty 'Thank you' to all of our donors and encourage those who can to join them.

Exercise your heart this holiday season!

The holidays will soon be here and many of us are trying to figure out what to give those hard to buy for people on our list. This year, why not make a contribution to Alpha-1 Canada by making a donation in the name of an outstanding parent, teacher or friend. Making a donation in someone else's name is also a great way to both support a charity and honor a deserving individual.

Alpha-1 Canada has a number of programs and services that can benefit from your support:

- ◆ the patient support hotline at 1-888-669-4583 is available to patients and their caregivers for information on both a regular and emergency basis;
- ◆ the medical advisory board meets regularly providing the board of directors and staff with information on the latest advances in research and treatments;
- ◆ the web site (www.alpha1canada.ca) is continually updated with the latest information;
- ◆ this monthly newsletter keeps patients and their caregivers up-to-date on current events and offers stories about fellow Alphas;
- ◆ in addition to the web site and newsletter, printed materials are distributed and are available to anyone, printing and postage are very expensive;

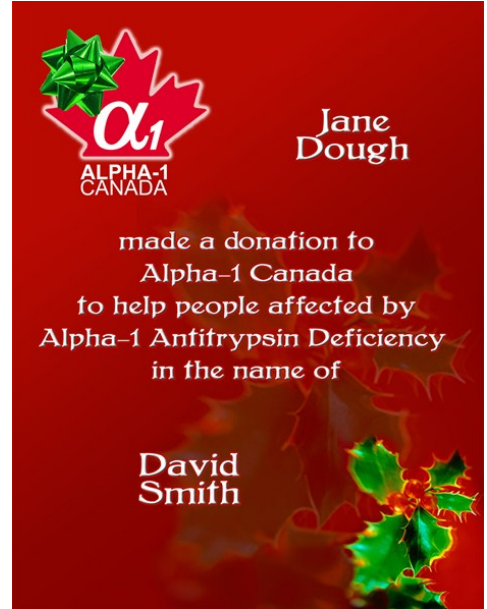
◆ patient support is offered through a variety of methods including e-mail, the hotline and a number of support groups;

- ◆ through Alpha-1 Canada's membership in the Canadian Organization for Rare Disorders (CORD) we keep informed about government activities related to healthcare and drug coverages and participate in supporting those initiatives that will benefit the Canadian Alpha-1 Community.

These are just a few of the programs and services you can support and help others during this time of love and giving and receive a tax write-off in the process. We will send you a gift card for easy giving.

People often tell us that they can't afford to give much and that is certainly understandable, especially in these difficult economic times. But it doesn't take much to make a difference.

A donation of \$5 can pay the toll costs to make sure the parents of a newly diagnosed baby can talk to someone about what to expect and learn that although their family's lives will change, there is hope. Just \$10 can make sure two more doctors receive information on when to



suspect a COPD or asthma patient may have Alpha-1 and how to get testing done. If two people were to donate \$100 each we could hold another Drop-in Meeting with a guest speaker to answer many questions and the recording of that meeting could help hundreds more who listen to the recording on our website.

Some of our donors use www.CanadaHelps.org to make their donations every month and multiply the good they do by twelve. CanadaHelps.org can accept credit card donations on our behalf on a one-time basis or even make those donations happen monthly without you having to log in over and over.

Knowing that a gift is a selfless contribution that made someone else's life brighter can put the season in perspective for you and the person in whose name you make a donation, perhaps even your own.

Interesting Research

The following are summaries of abstracts of recent studies of Alpha-1 and COPD.

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. Please note that Dr. Bourbeau (pictured above), who is a member of Alpha-1 Canada's Medical Advisory Board, is a co-author of the study in the second abstract.

Hydrolysed Formula Is a Risk Factor for Vitamin K Deficiency in Infants With Unrecognised Cholestasis.

van Hasselt P, de Vries W, de Vries E, Kok K, Cranenburg E, de Koning T, Schurgers L, Verkade H, Houwen R. *Department of Pediatrics, Wilhelmina Children's Hospital, University Medical Center Utrecht, The Netherlands †Department of Pediatrics, Beatrix Children's Hospital, University Medical Center Groningen, Groningen, The Netherlands ‡Department of Medicine, Division of Gastroenterology and Hepatology, Radboud University Medical Center, Nijmegen, The Netherlands §Cardiovascular Research Institute and VitaK, Maastricht University, The Netherlands.

Journal of Pediatric Gastroenterology and Nutrition. 2010 Nov 3. [Epub ahead of print]

Abstract

Abstract

OBJECTIVES: Vitamin K deficiency (VKD) may cause life-threatening haemor-

rhages, especially in breast-fed infants with unrecognised cholestasis. Interestingly, hypoallergenic formulas appear overrepresented in reported cases of VKD bleeding (VKDB) in formula-fed infants. We therefore assessed whether the risk of VKD in formula-fed infants with cholestasis is associated with hypoallergenic formulas.

PATIENTS AND METHODS:

Infants born in the Netherlands between January 1991 and December 2006 with cholestatic jaundice due to biliary atresia (BA) or to alpha-1-antitrypsin deficiency (A1ATD) were identified in the Netherlands Study Group for Biliary Atresia Registry and the A1ATD registry, respectively. The relative risk (RR) of VKDB in patients with BA or A1ATD was calculated for different formula types. The influence of prior or ongoing breastfeeding on the RR of VKDB was also assessed and was not significantly associated with VKD.

RESULTS and CONCLUSIONS:

A total of 179 infants with either BA (139) or A1ATD (40) were included. Risk factor analysis revealed that infants receiving hydrolysed, especially whey-based, formula, had a strongly increased risk of VKD compared with infants receiving regular formula. Infants with cholestasis receiving (whey-based) hydrolysed formula are at increased risk of developing VKD, compared with infants receiving regular formula. Because VKD may lead to serious haemorrhages, infants receiving whey-based hydrolysed formulas may need additional vitamin K supplementation.

Effects of written action plan adherence on COPD exacerbation recovery.

Bischoff EW, Hamd DH, Sedeno M, Benedetti A, Schermer TR, Bernard S, Maltais F, Bourbeau J. Department of Primary and Community Care, Radboud University Nijmegen Medical Centre, Nijmegen, The Netherlands.

Thorax. 2010 Oct 30. [Epub ahead of print]

Abstract

Background: The effects of written action plans on recovery from exacerbations of chronic obstructive pulmonary disease (COPD) have not been well studied. The aims of this study were to assess the effects of adherence to a written action plan on exacerbation recovery time and unscheduled healthcare utilisation and to explore factors associated with action plan adherence.

Methods: Exacerbation data were recorded for 252 patients with COPD who received a written action plan for prompt treatment of exacerbations with the instructions to initiate standing prescriptions for both antibiotics and prednisone within 3 days of exacerbation onset. Following the instructions was defined as adherence to the action plan.

Results: From the 288 exacerbations reported by 143 patients, start dates of antibiotics or prednisone were provided in 217 exacerbations reported by 119 patients. Adherence reduced exacerbation recovery time, but did not affect unscheduled healthcare.

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Michigan Focuses Attention on Alpha-1

November is now officially Alpha-1 Antitrypsin Deficiency Month in Michigan.

The goal of Alpha-1 Antitrypsin Deficiency Month is to increase public awareness of the condition through screenings, conferences and events to encourage early detection and also organ donation.

To read the full article, go to : <http://www.prnewswire.com/news-releases/alpha-1-antitrypsin-deficiency-month-in-michigan-focuses-attention-on-debilitating-genetic-disorder-106971538.html>



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!

Interesting Research

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Factors associated with an increased likelihood of adherence were influenza vaccination, cardiac comorbidity, younger age and lower FEV1 as percentage predicted.

Conclusions: This study shows that adherence to a written action plan is associated with a reduction in exacerbation recovery time by prompt treatment. Knowing the factors that are associated with proper and prompt utilisation of an action plan permits healthcare professionals to better focus their self-management support on appropriate patients.

Oxidation of Z {alpha} 1-antitrypsin by Cigarette Smoke Induces Polymerization: A Novel Mechanism of Early-onset Emphysema.

Alam S, Li Z, Janciauskiene S, Mahadeva R. Medicine, University of Cambridge, Cambridge, United Kingdom.

American Journal of Respiratory Cell and Molecular Biology. 2010 Oct 22. [Epub ahead of print]

Abstract

RATIONALE: The acceleration of chronic obstructive pulmonary disease by cigarette smoke in individuals with severe genetic deficiency of α 1-antitrypsin exemplifies the critical importance of gene-environmental interactions to the development of COPD.

OBJECTIVES: We investigated the molecular basis for the interaction between

Z-AT and cigarette smoke. **METHODS:** Eight to ten week old female mice transgenic for normal (M-AT) or Z-AT on CBA background were exposed to 4 1R3F cigarette smoke (CS) daily for 5 days. Z-AT was purified from plasma and exposed to cigarette smoke extract and assessed for the development and temporal relationship between AT conformers.

CONCLUSION: Transgenic mice for Z-AT developed a significant increase in pulmonary polymers following acute CS exposure. There were also increased neutrophils in CS-Z lungs. Our data show that cigarette smoke accelerates

polymerization of Z-AT by oxidative modification, which in so doing further reduces pulmonary defense and increases neutrophil influx into the lungs. These novel findings provide a molecular explanation for the striking observation of premature emphysema in ZZ homozygotes who smoke, and raise the prospect of anti-oxidant therapy in Z-AT related COPD.

COPD and cognitive impairment: the role of hypoxemia and oxygen therapy.

Thakur N, Blanc PD, Julian LJ, Yelin EH, Katz PP, Sidney S, Iribarren C, Eisner MD. Department of Medicine.

International Journal of Chronic Obstructive Pulmonary Disease. 2010 Sep 7;5:263-269.

Abstract

BACKGROUND: Several studies have shown an as-

sociation between chronic obstructive pulmonary disease (COPD) and cognitive impairment. These studies have been limited by methodological issues such as diagnostic uncertainty, cross-sectional design, small sample size, or lack of appropriate referent group. This study aimed to elucidate the association between COPD and the risk of cognitive impairment compared to referent subjects without COPD. In patients with established COPD, we evaluated the impact of disease severity and impairment of respiratory physiology on cognitive impairment and the potential mitigating role of oxygen therapy.

METHODS: We used the Function, Living, Outcomes and Work (FLOW) cohort study of adults with COPD and referent subjects matched by age, sex, and race to study the potential risk factors for cognitive impairment among subjects with COPD. Cognitive impairment was defined as a Mini-Mental State Exam score of <24 points. Disease severity was using Forced Expiratory Volume in one second (FEV(1)); the validated COPD Severity Score; and the BMI (Body Mass Index), Obstruction, Dyspnea, Exercise Capacity (BODE) Index. Multivariable analysis was used to control for confounding by age, sex, race, educational attainment, and cigarette smoking.

CONCLUSION: COPD is a major risk factor for cognitive impairment. Among patients with COPD, hypoxemia is a major contributor

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INTERESTING RESEARCH

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and regular use of home oxygen is protective. Health care providers should consider screening their COPD patients for cognitive impairment.

Asthma and COPD in Alpha-1 Antitrypsin Deficiency. Evidence for the Dutch Hypothesis.

Eden E. Director Pulmonary, Critical Care and Sleep Medicine, St Luke's Roosevelt Hospital Center, Columbia University, New York, NY.

COPD. 2010 Oct;7(5):366-374.

Abstract

ABSTRACT: This review summarizes the current information on the relationship between severe alpha-1 antitrypsin deficiency (AATD), asthma and COPD. AATD is a genetic predisposition to the development of early COPD in susceptible individuals and reduction in known factors that enhance lung function loss is the paramount aim of management. Asthma is one controllable condition that leads to the accelerated decline in lung function. Current literature indicates that asthma signs and symptoms are common in those AATD with or without COPD and that bronchodilator response is a risk factor for FEV(1) decline. Furthermore AATD itself predisposes to airway hyperresponsiveness, an essential ingredient for reversible airflow obstruction. In the absence of well-characterized markers to distinguish COPD from asthma, clinical diagnosis leads to a delay in the recognition that asthma

symptoms such as wheezing can be an early manifestation of COPD in AATD. In addition failure to appreciate asthma overlap in AATD may lead to inadequate suppression of airway inflammation leading to the development of airflow obstruction. The implications of this are discussed as are potential approaches and recommendations for treatment.

Alpha 1-antitrypsin deficiency-related alleles Z and S and the risk for Wegener's granulomatosis.

Mahr AD, Edberg JC, Stone JH, Hoffman GS, St Clair EW, Specks U, Dellaripa PF, Seo P, Spiera RF, Rouhani FN, Brantly ML, Merkel PA; for the WGER Research Group. Boston University School of Medicine, Boston, MA.

Arthritis and Rheumatism. 2010 Sep 8. [Epub ahead of print]

Abstract

OBJECTIVE: Alpha 1-antitrypsin deficiency (AATD), a genetic disease that predisposes to emphysema and liver cirrhosis, may also be a determinant of susceptibility to Wegener's granulomatosis (WG). Several, mainly small, case-control studies reported that 5-27% of patients with WG carried the AATD Z allele. It is not clear whether the S allele, the other major AATD variant, also contributes to the risk of developing WG. We investigated the relationship of the AATD Z and S alleles with the risk of developing WG in a large, well-characterized cohort.

METHODS: We studied the

distribution of the AATD alleles Z and S in 433 unrelated Caucasians with WG and 421 ethnically-matched controls without disease.

RESULTS: Among the patients with WG, the allele carriage frequencies of Z and S were 7.4% and 11.5%, respectively. The odds ratios (OR) for developing WG associated with allele carriage was 1.79 (95% CI, 0.95-3.44) for Z, 1.59 (95% CI, 0.97-2.62) for S, and 1.68 (95% CI, 1.13-2.58) for Z and/or S. Compared to the normal MM genotype, the OR for MZ or MS genotypes was 1.47 (0.98-2.22), and the OR for ZZ, SS or SZ genotypes was 14.58 (2.33-infinity).

CONCLUSION: Both Z and S alleles display associations with WG risk in a co-dominant genetic model. These findings strengthen the evidence for the causal link between AATD and WG susceptibility.

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



WORLD COPD DAY

This year's World COPD day was held on Wednesday, November 17, 2010 in over 50 countries. The theme of this year's event was "2010 - The Year of the Lung: Measure your lung health - "Ask your doctor about a simple breathing test called spirometry."

From a virtual exercise event organized by the Lung Association of Saskatchewan to a COPD Awareness week building event from the Lung Association of Québec, activities took place around Canada and the world.

To see a list of local and international activities, as well as an archive of past years' events, visit : <http://goldcopd.com/WorldCOPDDay-inYourCountry.asp?l1=5&l2=0>

Alpha-1 Antitrypsin Deficiency Canada Inc.

1638 Northway Avenue,
Windsor, Ontario N9B 3L9

Phone/Tél. : 519-258-1444
Fax/Télé. : 519-258-1614
Toll Free/Sans frais : 1-888-
669-4583
E-mail/Courriel :
info@alpha1canada.ca



Visit us on the web
at alpha1canada.ca

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

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

The development and implementation of a Canada-wide regulatory framework for rare disorders – Promises kept?"

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To call in, simply dial 1.800.508.7891 and enter your participation code: *9056145591*. The asterisks (*), before and after the meeting number, are required.

If you are unable to attend, but have questions you would like to ask Dr. Wong - Rieger, please contact Alpha-1 Canada at 1-888-669-4583 or

vanessa.mclaughlin@alpha1canada.ca before December 1st, 2010.

Responses to your questions will be posted on our website.