

SHARE IN LIFE NEWS

AUTUMN 2011

INTRODUCTION FROM THE CHAIRMAN



Firstly, I would like to introduce myself and two new members – Dr Marie Johannesson and Peter Tinholt. Peter and Marie bring with them a great mix of skills and experience to add to our Board with Jane Drumm and John Parsons remaining on the new Board.

We live in challenging times. For a Foundation such as ours, a worldwide recession is a tough time to attract

funding to increase our impact. Combine this with earthquakes and you would think the task impossible. However, in tough times as it is with living with CF, often it is about getting up each day and simply 'getting on with it'. We know CF is a complex, illness and despite improved knowledge, it still abbreviates lives the world over. Often for families and PWCF experiencing all the challenges of CF, progress toward adequate control over the disease, can seem too slow.

The good news, is that there are exciting developments pending and I cannot remember a time that has held such hope for our community. In the past year, we have been working on implementing our clinical benchmarking database - PORT CF. Once live, it will give clinical teams another tool to understand the CF community. They will be able to accurately measure how our community is tracking in terms of clinical results and patient outcomes. Ultimately, these results will not only compare clinics within NZ, but our clinical teams will be able to compare our results to clinics in the USA, Australia and Britain.

Once the results are released, quality improvement processes occur, which over time, lift standards and improve results.

It is disappointing to report that the roll out of Port CF has been affected by the Canterbury earthquakes. Our PORT CF server is located at the Canterbury DHB in Christchurch and the redeployment of DHB computer staff to fix other systems, has taken them away from attending to our database.

This is not ideal, but there is little to be done except be patient.

In February, we saw the enrolment of a small number of NZ-based people with CF into a phase two US CF Foundation trial 'Vertex 809 and 770'. Considering there were only 22 sites selected throughout the world and NZ got two, this is a major achievement. Congratulations to the Auckland and Christchurch clinics. Hopefully it will be the start of attracting many more trials here. More on the Vertex trial later in this issue.

Earlier this year, I travelled down to the University of Otago to meet with a renowned NZ researcher Prof Iain Lamont. For many years he and his student researchers have been looking at an old foe of CF lungs, *Pseudomonas Aeruginosa*. To progress his research focus further, Prof Lamont submitted an application for funding and the SILF, in partnership with Cure Kids, has approved it. This is great news and we wish him and this team all the best. (Below is an article about Prof Lamont and his team and we look forward to updating you on their progress.)

Whilst all of these developments are positive and moving us in the right direction, we know that in order to really make a significant difference to CF in NZ, we need help.

We are on the brink of massive change here in NZ. Our early work to get our CF database working is nearly complete and the results it will produce will drive a patient study emphasis.

Sadly our NZ people with CF do not have the same access to some international gold standard medications and treatments. Pharmac actively controls our access based on a funding model that disadvantages rare chronic conditions such as CF.



Chairman's Introduction Continued

Whilst it would be nice to think that our CF population has not been affected by this policy, we may find that the reality is different. If we discover that there are gaps in where we stand against other first world CF countries, we will need targeted patient studies to understand this as well as education and a lobbying push to improve things. This will be a lot of work, taking money and people.

If there was ever a time to support your Foundation it is now so over the coming months we will be approaching those within our CF family to help us to build our capacity in various ways. The planning and refocusing of our Foundation and CF NZ is currently taking place. Over the last few months, the CF NZ Board has been preparing for a strategic planning weekend, the results of which will be announced in the next few months.

In the meantime, stay tuned and stay optimistic. Things can only get better. The years of standing on the side-lines, watching CF run its course and feeling there is little we can do here in NZ will soon be over. We are nearly at the point that we can all turn our hope into action. These are indeed exciting times.

Andrea Miller



INTRODUCING PROFESSOR IAIN LAMONT

Dr Lamont has an extensive track record in the field of bacterial infectious disease, molecular biology, biochemistry and genetics. His research has focused on pathogenicity of *Pseudomonas aeruginosa* and in particular, the role of iron uptake. This has resulted in identification and characterisation of multiple genes and enzymes that contribute to these processes. It has also involved an intensive analysis of gene expression and of regulatory mechanisms that control iron transport pathway. His research has extended into analysis of *Pseudomonas* iron uptake pathways in cystic fibrosis and in particular, the development of novel processes for examining the activities of *Pseudomonas* genes as they occur in CF.

Dr Lamont's research is internationally recognised and highly cited, resulting in his involvement as an international specialist contributing to analysis of the genome sequence of *P. aeruginosa* with responsibility for genes involved in iron uptake and metabolism as well as regular invitations to international meetings to describe his research. Dr Lamont is also the Director of the Genetics Teaching programme at the University of Otago.

Career Background: Originally Scottish (but now proud to be a New Zealander!) with a BSc (Hons) in Microbiology, University of Edinburgh (graduated 1980) and a PhD University of Oxford (bacterial genetics) (graduated 1983). He has undertaken postdoc research (molecular biology) University of Adelaide (1984-87). He moved to Otago in 1987.

Prof Lamont's Team CF Research Focus

Prof Lamont believes that "our approach should give a new level of understanding about how *Pseudomonas* in the lungs of people with CF reacts to antibiotics. I am very excited that we might be able to take some of the basic research we have done in the lab, and translate that into something that could end up helping these patients".

The Shares in Life Foundation are very excited to be supporting this project with a grant from our fund, in partnership with Cure Kids



Professor Lamont's Team

Left to right. Lois Martin (senior research technician), Eleanor Middleton (studying for BSc (Hons), Katy Booth (studying for MSc), Becky Edgar (studying for PhD), Leo Germoni (studying for PhD), Georgi Hampton (research assistant), Prof Iain Lamont

You can read more detail about Professor Lamont's research by visiting this link

<http://biochem.otago.ac.nz/professor-iain-lamont/>



NEWS FROM THE STATES—VERTEX TRIALS NEW DRUGS

VX-770 and VX-809 are two individual drugs that have been evaluated in separate clinical trials, and are currently being studied in combination. VX-770 is designed to allow the CFTR protein located at the cell surface to work correctly. In early 2011, Vertex released positive results of two Phase 3 clinical trials of VX-770 in people who carry at least one copy of the G551D mutation of CF.

One of these trials examined VX-770 in CF patients 12 and older, the other trial evaluated the drug in children age 6 to 11. In both studies, those taking the drug showed significant improvements in lung function, the chance of having a pulmonary exacerbation, weight gain and sweat chloride levels, compared with those on the placebo.

VX-809 is designed to move defective CFTR protein to its proper place at the cell surface. A Phase 2a clinical trial of the drug in patients with the most common mutation of CF, Delta F508, showed positive results.

In 2010, Vertex launched a clinical trial to evaluate multiple combinations of VX-770 and VX-809. The three-part trial will test the safety and tolerability of the two drugs in individuals with two copies of the most common CF mutation, Delta F508. The Delta F508 mutation creates a defective CFTR protein that does not move to its proper place at the cell surface. Researchers believe that by using VX-809 in combination with VX-770, CFTR may work the way it should.

VX-809 is designed to move the CFTR protein to the cell surface; VX-770 is designed to allow CFTR located at the cell surface to function correctly. Vertex expects to have data from the first part of the combination trial of VX-770 and VX-809 in the middle of 2011.

The CF Foundation worked with Vertex to discover VX-770 and VX-809, and has provided substantial scientific, financial and clinical support throughout the development process, including an approximately \$75 million investment.

The new program will support development of a potential new drug called VX-661, designed to treat people with the most common genetic defect in CF, the Delta F508 mutation. Nearly 90 percent of people with CF in the United States have at least one copy of this mutation.

VX-661 is known as a "corrector" and aims to move the defective CF protein to its proper place at the cell surface. Another corrector, known as VX-809, is already in clinical trials. By developing multiple correctors, the Foundation increases the chances of bringing new therapies to the CF community as quickly as possible.

"This new agreement will further leverage the successful collaboration with Vertex to accelerate the discovery and development of new drugs to treat a wide variety of CF patients," said Robert J. Beall, Ph.D., president and CEO of the CF Foundation. "Given the recent announcement of promising data of other compounds in the CF pipeline, we're optimistic that the CF Foundation is on the right path to fundamentally change the treatment of CF by targeting the cause of the disease."

Potentiator - VX-770, known as a CFTR potentiator, aims to increase the function of defective CFTR proteins by increasing the gating activity, or ability to transport ions across the cell membrane, of CFTR at the cell surface.

Corrector - In people with the F508del mutation, CFTR proteins do not reach the cell surface in normal amounts. VX-809, known as a CFTR corrector, aims to increase CFTR function by increasing the trafficking, or movement, of CFTR to the cell surface.

CFF link to webinar on Vx 770/809 <http://www.cff.org/LivingWithCF/Webcasts/>



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HOW CAN YOU HELP?

New research opportunities present themselves all the time and the Cystic Fibrosis Association of New Zealand is committed to remaining ready to respond with funding when our talented scientists and clinicians come to us for help with research projects. Our Mission is clear—to work toward a better quality of life for all people with Cystic Fibrosis, through research into better treatment and ultimately, a cure.

Your help in this process is vital. Please help us to ensure that research continues to find better ways of dealing with the effects of this life-limiting condition.

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