SUBMISSION FOR THE SELECT COMMITTEE INTO FUNDING FOR RESEARCH INTO CANCERS WITH LOW SURVIVAL RATES

April 2016 changed my life forever. It was the time when I became not just a wife, mother, grandmother and friend but also a neuroendocrine cancer patient. The news affected not only myself but the lives of all those connected to me. As we cried we began to look for hope, for a future, for treatment and cure. There was none. Rare cancers in our relatively small national population are underrepresented in research funding. There will presumably always be too few sufferers, despite a growing prevelance, to constitute a statistically significant cohort with which to provide proof of viable research evidence and treatment options. In a model which is weighted heavily toward economic benefits, taking into account positive outcomes for many over positive outcomes for the few, people with rare cancers (and other rare medical conditions) can never win. They will forever be the forgotten Australians with no voice to rally support or funding to fight for their rights – the right to equity and access to treatment which can ease their suffering, relieve their symptoms and prolong their lives. Researchers, doctors, nurses and technicians who should be utilizing their not inconsiderable talents in undertaking active research have no choice but to apportion much of their energy and time to raising research funds, completing seemingly endless paperwork and complying with out-moded, tangled, repetitive bureaucratic red tape with little or no hope of their funding requests ever being met. Families and patients are likewise required to advocate on their own behalf and to raise funds to support research, engendering much distress and stress, rather than spending quality time together.

NHMRC FUNDING MODEL

My knowledge of funding models was zero when I began to search for information in this regard. The internet revealed that theoretically anyone can make a submission for research funding. The reality seems to be very different. Submissions are time limited to just six weeks with a complex array of committees, an Expert Advisory Group, Peer review and countless pages of nested compliance prerequisites to read, absorb and address. Hours of my life disappeared as I struggled through compliance lists and branching sub-pages. While I could find documented funding for neurological and neuroscience ($188.3 million) on the NHMRC online site I could find no breakdown of how this had been apportioned. Likewise for cancer research at $191.9 million up to 4 April 2016. Drop down tables of grant approvals were in minute print and difficult to read. Attempting to scroll through them to identify and collate NETs grants was impossible. Researchers compelled to comply with such multitudinous red tape must waste many hours which could be more productively used doing what they do best – research, not paper shuffling. Often they must find themselves competing for limited funding against the very peers who are meant to assess their research proposals and against high-profile, well known and supported disease research groups with greater numbers to lend weight to their arguments. Smaller groups are seriously disadvantaged in this race for available money. Too few in number to be statistically significant in the major disease stakes we patients are sidelined and disadvantaged in comparison. Non-quantifiable issues seem to be ignored – human rights to equitable access to drugs, treatments and the research on which medical opinions and decisions are based. Not all are equal in our democratic society. A per capita funding model is not evident. Those who wield the most power achieve the greatest returns. Those with rare diseases are discriminated against in relative terms. I know of a young pancreatic cancer researcher who has had to abandon his work to find other

employment to support his family as the erratic, unreliable short term nature of the funding of his project proved insufficient to provide wages for the team.

OBSTACLES TO RUNNING CLINICAL TRIALS

- **Lack of funding combined with inconsistency of funding means that clinical trials are seldom available. For example CONTROLNETS clinical trial for midgut and pancreatic NETs (neuroendocrine tumour) patients. Stage I is funded largely by the Unicorn Foundation via public donation, fundraising events and some corporate sponsorship. With a goal of $200,000 to begin research they achieved more than $250,000 which is a goodly sum of money to be contributed. One would expect such work to be rewarded and encouraged but Stage II funding has once again been denied. Relatively few patients can have the chance to participate as places are very limited. Royal North Shore Hospital, Sydney, has only 10 such places for more than 120 listed patients. I am one of the fortunate few to have a chance to fight back and possibly achieve remission, contributing in a small way to a research database along the way. Without the advocacy and support of my local oncologist and RNSH NETs nurse, I would have been totally unaware of this possible opportunity. This chance to tackle NETs restores dignity, enhances self-worth and improves mood, all non-quantifiable benefits of research participation.**
- **Awareness of the availability of clinical trials which you can apply to participate in is very low. Little or no funding is available to advertise trials. Research teams do not have the time to actively seek out suitable candidates or to notify every patient that a clinical trial is about to begin. Knowledge of current research is very hit and miss from the patient viewpoint, relying on word-of-mouth, internet searches and your particular treating doctor being linked in to research centres and/or having the time and interest to pursue possible options for their patients. With the current pressure of numbers on local doctors and demand for quantitative performance few practitioners have the luxury of such time.**
- **Possible reluctance of patients to participate in clinical trials could be linked to lack of time and funding. It takes considerable time to calmly and clearly explain the aim of trials, how they will work and what risks may be involved. Such time is not funded. Hours were spent educating, discussing, explaining and reinforcing ideas in regard to NETs with me – much longer than is allowed in usual consultation times. All of this extra work is attended whilst undertaking tests, scans, infusions and physical examinations and is on top of normal workload. The organisers and speakers at the NETs symposium in Melbourne on 6 November 2016 gave up their Sunday leisure time to help educate others – a sacrifice which is neither recognised nor rewarded by the Federal Government. Only attendees, mainly patients and their support persons, appreciated the time and effort contributed by these wonderful people. Funding 'talk time' would reassure patients, enhancing their understanding of their disease and contributing confidence toward entering research and clinical trials. Running clinical trials across pooled national groups could also help to redress the low numbers which can be funded. Some of this cross-national dialogue and research is already happening but there could and should be so much more.**

LOW SURVIVAL RATE

NETs has an almost 100% mortality rate. No-one can dispute this. Only very few are fortunate enough to be diagnosed with just one resectable tumour. Most people are diagnosed far too late and already have multiple metastatic tumours many of which are non-resectable. Lack of specific symptomology, non-diagnosis or multiple mis-diagnosis leave too great a time lag before

visualisation of tumours can take place and treatment begin. Hence it is essential that funding is found to develop an early warning system – a diagnostic blood test for tumour markers would alert potential suffers and their doctors to the need for further investigations. Work is under way to find this marker but there are too few researchers and too little funding to make sustained and coherent progress toward this aim. Early diagnosis would not only save lives but reduce the cost of higher level interventions as the disease progresses. Patients would have their health improved much earlier and could return to the workforce and other productive activities like volunteering and child-minding, reducing the burden of cost for their care and endowing them with positive input into society. Cure is not the ultimate or only criteria for success. Enabling independence in financial and independent care spheres would prolong workforce participation; less dependence on mood enhancing medications, support services and psychological counselling; increased participation in societal and familial roles not just for patients but also their carers and other family members. Independence means real monetary savings in the longer term. The Australian Code for the Responsible Conduct of Research (Box A.1) defines research as obtaining "improved insights…the creation, development and maintenance of intellectual infrastructure". Nowhere in this document does it allude to research having to produce a cure. Knowledge and understanding are the only parameters utilised. Why then is cure and/or survival rate mooted as significant in determining the value of research?

LACK OF IMPROVEMENT

The reaction of most people when you say that you have NETs is that you are doomed, that all is lost and preparing for death is all that you can do. "I'm so sorry" applied with a sympathetic pat to my shoulder has been my general experience from many doctors, family members and friends. When you Google NETs the information that comes to your screen is fear engendering and largely outdated. On top of the shock of diagnosis comes the slow devastating realisation that although treatments exist you may well be refused access to them. Drugs are approved for one type of NET but not others. PRRT which has been proven effective over many years is doled out on an individual basis "on compassionate grounds". How is it compassionate to restrict and withhold treatment modalities which may elicit positive outcomes for Australians who happen to have a rare disease? Improvement will not come when we are denied a chance to try. We are not just "patients" but also human beings. Being denied the right to fight for life is not compassion nor is it empathy but it is cruelty of a most heinous kind. Where is the recognition of our humanity, our right to make our own decisions, the much touted Aussie concept of "a fair go"? We do not seek handouts to no purpose just support in our fight. The High Court of Australia both defines and upholds the right of people to give informed consent to their treatment. I would argue that regulatory bodies denying access to available treatments is a breach of this fundamental legal right and is both immoral and unethical. Research combined with new technologies has most definitely made a quantifiable improvement. Genetic research, smart drugs, nuclear medicine, improved imaging and microscopy have all contributed to a greater understanding of NETs. Our brilliant, determined researchers are among the world's best but who ever hears of them? Prior to my diagnosis I had no idea this rare disease even existed let alone that our peer-recognised teams were leaders in their fields. Life cannot always be measured in its' greatness by achievements, money, property owned or length of endurance. There are many subtle, non-measurable qualities which enhance our daily existence and combine to create a life both well-lived and enjoyed. Death will claim us all – it is life's only certainly. Whether our end is due to a rare disease or no is utterly

irrelevant to our quality of life and the contributions we may make to our loved ones, society and planet earth. Being able to access treatment which allows us to be mobile, to socialise, to care for ourselves and our loved ones, to attend those special events and cherish special moments such as the birth of a child, a marriage or graduation, are invaluable and unforgettable. They are moments in time that bind us to each, and from generation to generation, endowing us with social cohesion, happiness and familial caring. No amount of money can buy these feelings but research funding can help us to stay around long enough to experience them. Time flows on inexorably but just a few days, months or years extra can make such a difference, not just to those who will die but to those who remain. Happy memories sustain, enrich and empower us. Research can help us leave such a legacy behind.

STRATEGIES FOR IMPROVEMENT

Education and support are vital. Initial diagnosis is shocking. Lack of reliable information more so. There is one online dedicated NETs nurse for all of Australia where there are over 10,000 known NETs patients. We need many more. It is unknown exactly how many we, the patients, number. NETs is highly diverse with many different types of presentation under one umbrella diagnosis. There is one NETs nurse at Royal North Shore Hospital – nowhere near enough for over 120 people. There are no NETs nurses on the Central Coast of NSW where I live. There are NETs support groups at Royal North Shore Hospital and the Adventist Hospital, Wahroonga both more than an hours drive away down the busy M1. There are no support groups on the Central Coast. Imagine: night - you are home self-injecting octreotide in the bathroom while you wait for your symptoms to become severe enough to enter the SHINE programme. Dizzy, nauseous, shaky, crampy abdominal pain. Afraid. No-one to ring. No-one will come. Just cross your fingers and hope you get by. SHINE sponsored by Norvartis provides nurse visits at home to give injections of Somatostatin. This nurse is not NETs aware. You are still alone with your symptoms, unanswered questions and fear. Rural and remote patients are even more alone and underrepresented in the national NETs patient count.

OTHER RELEVANT MATTERS

To die knowing that everything that could be done was done and that you fought your hardest matters. The legendary Ozzie spirit of 'giving it your all' pervades our culture and our lives. Right now Australians with rare diseases are being denied access to drugs and treatments which could enhance and lengthen our lives. We are being denied that chance to fight for life. Research can give us that chance. Hope is defined and denied by dollars and cents or the lack thereof. Even in times of fiscal stringency we can do better. Money is wasted on trivialities such as corporate signage and uniforms, colour harmonics consultants and wall art organisers. People without hope do not care about such things, about image creating or appearances – they just want treatment options and to live. Real priorities must include funding research, developing drugs and treatments, education, patient and family support, recognition of reputable valid overseas research and drug accreditation. It is not necessary to duplicate research just to make it Australian. Many integrated, co-operative research entities operate in our multi-national world. Why not just accept their findings and safety standards and move on? Onward into new realms of research so absolute progress can be made. Just believe.

Sandra Joy Woods MN BSc BN Dip Ed

Ileal (midgut) neuroendocrine tumour (NETS) patient. Research participant 45 and Up Study; Defining NETs Study; CONTROLNETS Clinical Trial, Royal North Shore Hospital, Sydney, NSW