The Exceptional Responders Program: Malignant pleural mesothelioma sub-group

The Garvan Institute, together with The Kinghorn Cancer Centre, has launched an exceptional responders program across all solid-organ cancers, with a sub-group focused on malignant pleural mesothelioma (MPM). Australia has one of the highest age-standardised incidence rates of mesothelioma in the world\(^1\), placing us in a unique position to contribute to the global understanding of therapeutic approaches to the disease.

What is an Exceptional Response?

There are many variables of treatment response (drug, host, cancer) and cases are often multifactorial. Generally, cases should meet one of these quantitative criteria:

- CR or PR, where <10% of patients are expected to respond
- CR or PR that lasts > 3 times the median / expected PFS
- Best response, PD where >90% of patients expected to respond

For the MPM sub-group, we are recruiting patients to one of three categories of response:

- Indolent disease i.e., non-progression in the absence of anti-cancer therapy
- Exceptional response to standard chemotherapy
- Exceptional response to immunotherapy

Following referral, each case will be qualitatively assessed by our MPM sub-committee for inclusion

Case examples:

**Indolent disease:**

78yo M, epithelioid MPM 2016. L pleural effusion 2011; L thoracentesis → atypical mesothelial proliferation. Represented 2016 with recurrent effusion → Bx MPM. No therapy. Sustained SD since.

**Chemotherapy responsive:**

63yo F, epithelioid MPM 2014. L pleural effusion, mediastinal LN on CT 2014; VATS and pleurodesis → Bx MPM. 6 cycles carboplatin and pemetrexed, then maintenance pemetrexed. Sustained PR since.

**Immunotherapy responsive:**

66yo M, epithelioid MPM 2015. Initial radiotherapy then chemotherapy → best response PD. Switched to pembrolizumab July 2016. Initial PD, ceased after 6 cycles due to clinical deterioration. CT 7 months later showed near CR, sustained.
How do I refer a patient?
If you have a patient you think may be suitable, you should discuss it with the patient and send us a standard referral letter OR complete the referral template (available online):

- Email: patient@exceptionalresponder.com.au
- Fax: 1800-PATIENT
- Via the website: www.exceptionalresponder.com.au

We will contact the patient and arrange a Telehealth consultation for consent. We require a one-off blood sample (2x10mL tubes) and access to archival tissue.

What are we doing with the cases?
Blood will be processed and bio-banked for future DNA and RNA analysis. Tumour analysis may vary, but will include DNA sequencing and targeted analysis based on individual response and tissue quality. Sequencing data will be made available for future researchers in a public repository.

Find out more: www.exceptionalresponder.com.au

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