Published on *The Royal Marsden BRC* (https://www.cancerbrc.org)

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Rare talents

05 December, 2019

There are more than 200 types of cancer that can be classed as uncommon ? and experts at The Royal Marsden and the ICR are at the forefront of research into these diseases

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Rare and less common cancers ? those other than the four most common malignancies of breast, prostate, lung and colorectal ? account for 46 per cent of all cases but 54 per cent of deaths. It is this disparity between diagnosis and mortality rates that makes the research into these 200 or so diseases at The Royal Marsden and the ICR so crucial. For example, in The Royal Marsden?s Neuro-oncology Unit, there are two main aspects to research. The first focus is on primary brain and central nervous system tumours. The second is on secondary cancers ? where it has spread to the brain or central nervous system from another area in the body.

?Compared with other types of cancer, these are rare and hugely varied,? explains Dr Liam Welsh, Consultant Clinical Oncologist in the Neuro-oncology Unit. ?We aim to treat patients to maximise their quality of life and overall outcomes. With treatment, some can have a long life expectancy, and sometimes even a cure.

?For primary brain and central nervous system tumours, we?ve seen improvements in diagnosis, thanks to better tools for classification. If we are able to distinguish the microscopic differences between tumours, we are able to give patients more appropriate and personalised treatment.

?Another improvement we?ve seen is that, thanks to advances made in imaging and radiotherapy technology, we can target tumours more precisely, which means fewer side effects for patients.?

Dr Welsh is working with the ICR and St George?s University Hospitals to build a preclinical programme of research. ?We?ve established a brain tumour tissue biobank in the NIHR Centre for Molecular Pathology,? he says. ?Analysing these samples will enable further characterisation of tumours at a molecular level. With the ICR, we hope this could lead to new ideas for treatment ? it really is an example of ?bench to bedside? and back again.

?It?s a unique scenario to have the NIHR BRC here at The Royal Marsden and the ICR. With the Oak Drug Development Unit, we are able to develop Phase I trials, which are so crucial with this specific tumour group.?

Treating cancer based only on what we see down the microscope isn?t good enough any more

Paediatric focus

All childhood cancers are considered rare, and some of these are a major area of research focus for the ICR. Most cancers occur in adults ? particularly older people ? as the genetic changes that cause the diseases are more likely to develop as we age. Because they affect such small numbers of children, childhood cancers are difficult to research, and developing treatments is a huge challenge. But the problem isn?t limited to finding new treatments. We know that sometimes the right treatments exist, but we need to use them more effectively.

Professor Louis Chesler, Professor of Paediatric Cancer Biology at the ICR and a consultant at The Royal Marsden, led a recent study that showed genetic testing is a powerful tool for picking out the best drugs for children with cancer that will extend and improve their lives. Using a gene panel test, researchers read the DNA sequence of 91 genes that drive the growth and spread of cancer from 223 children?s tumour biopsies to identify potentially ?targetable? mutations. The study found that half of these tumours had gene mutations that can be targeted using adult cancer drugs available either as standard treatments or via clinical trials.

?Our study has demonstrated that we have the scientific knowledge and technology to get children access to state-ofthe- art testing and treatments,? says Professor Chesler. ?And, because our testing currently only assesses a focused set of well-known and clinically meaningful mutations, it is more practical, more cost-effective and faster than looking at the whole genome.?

This is ostensibly great news. However, regulatory and funding barriers pose a challenge. Most children in the study could not access these drugs because there was no trial available in patients of their age, the treatments were unavailable to them on the NHS, or they were too ill to receive an experimental treatment by the time they were tested.

In September, Professor Janet Shipley, who leads the Sarcoma Molecular Pathology Team at the ICR, also called for better repurposing of adult drugs at the Childhood Cancer Conference ? the UK?s biggest annual gathering of the community of researchers and families searching for new treatments in childhood cancer.

Professor Shipley?s research focuses on rhabdomyosarcoma, a type of cancer resembling muscle tissue that primarily affects children and teenagers. ?We need better molecular markers and brand-new drugs for children with cancer,? Professor Shipley told her audience. ?But, very importantly, we also need to be repurposing existing drugs.?

Better understanding

It?s important to gain a thorough understanding of the genetic changes within childhood cancers. Professor Chris Jones, lead in Childhood Brain Tumour Biology at the ICR, is a worldleading expert in diffuse intrinsic pontine glioma (DIPG) ? an essentially untreatable childhood brain cancer with an average survival of just nine months ? and high-grade glioblastoma.

Professor Jones led a study gathering genetic data from 910 cases in 20 previously published analyses, and

157 new cases ? all of which focused on children or young adults up to the age of 30 with high-grade glioblastoma or DIPG. The results produced by this study are now considered the definitive dataset on these cancers, and the research community can access it via a public portal for use in designing new tests and treatments.

?Our study uncovered a wealth of new information about children?s brain cancers,? says Professor Jones. ?We found that tumours that have historically been lumped together under one diagnosis comprise many, remarkably different, diseases.?

While some children?s tumours are driven by a single genetic error in which genes are fused together, there are others where tens of thousands of genetic errors are involved. Thinking about targeted therapies for these tumours is therefore a complex process, and each disease needs to be assessed individually to understand what treatment will be appropriate.

?Treating cancer based only on what we see down the microscope simply isn?t good enough any more,? says Professor Jones. ?We need to start thinking about these as completely different cancers and diagnosing and treating them based on their genetic faults. It?s exciting that several types look like they could be clearly treatable using either existing drugs on the market, or other treatments under development.?

Rare cancers present huge challenges for research, drug development and access ? and leading-edge science is just one part of the story. Knowing more about the biology and genetics of these cancers will be essential ? but so will regulating drugs to ensure new treatments reach the patients who so need them.

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