Accelerating drug development for neuroblastoma

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An international forum of leading scientists from the UK, Europe and USA, funded by national charity Neuroblastoma UK, prioritises drug development and strategies for new treatment trials for children with neuroblastoma.

Neuroblastoma is the most common solid tumour in children after brain tumours with around 100 children diagnosed every year in the UK. Yet, despite vital investment in neuroblastoma research, only one new drug - Dinutuximab-beta - has been made available for children receiving front-line neuroblastoma therapy since the 1980s.

In the September edition of European Journal of Cancer, Dr Lucas Moreno from Vall d'Hebron Hospital, Barcelona, and a team of international scientific and clinical experts published an **agreed list of genetic targets and drugs that should be advanced for early-phase paediatric clinical trials,** following the second Neuroblastoma New Drug Development Strategy (NDDS) forum.

The second collaborative NDDS forum, funded by the national research charity Neuroblastoma UK, brought together a wider group of both North American and European academic researchers, patient advocates and industry representatives to evaluate and prioritise the development of new genetic targets and drugs for neuroblastoma, and to share the consensus.

The recent paper reports on agreed strategies to discover new and develop existing drugs, recommendations for drugs to rapidly enter paediatric clinical development and methods to optimise combination and immunotherapy treatment.

The key conclusions / outcomes from the meetings are:

- Pre-clinical and clinical data for 40 genetic targets and mechanisms of action were prioritised and drugs were identified for early-phase trials.
- Strategies to develop drugs targeting TERT, telomere maintenance, ATRX, alternative lengthening of telomeres (ALT), BRIP1 and RRM2 as well as direct targeting of MYCN are high priority and should be championed for drug discovery.
- Promising preclinical data suggest that targeting of ALT by ATM or PARP inhibition may be potential strategies. Drugs targeting CDK2/9, CDK7, ATR and telomere maintenance should enter paediatric clinical development rapidly.
- Optimising the response to anti-GD2 by combinations with chemotherapy, targeted agents and other immunological targets are crucial.

Dr Lucas Moreno, lead investigator and Clinical Director of Paediatric Oncology and Haematology at Vall d'Hebron Hospital, Barcelona said, "*We are very grateful for the pivotal support of Neuroblastoma UK. Whilst advances in neuroblastoma treatment have occurred, with eight of nine high-priority treatments now being evaluated in paediatric clinical trials, greater international collaboration is essential.*

"Neuroblastoma is a rare disease and a coordinated international effort is required to recruit sufficient numbers of patients for clinical trials. The NDDS meeting was a major advance - it produced a very important trans-Atlantic consensus for accelerating drug development and provides a clear understanding of where we should focus future clinical trials to deliver the best potential outcomes for our patients."

Tony Heddon, Chair of Trustees at Neuroblastoma UK said, "*This is a hugely important outcome in the Neuroblastoma New Drug Development Strategy for conducting research trials in neuroblastoma. It will accelerate bringing new drugs more rapidly to front-line therapy and could have truly global benefits. By funding vital research and supporting initiatives such as the NDDS, we can help develop new, more effective and kinder treatments for children with neuroblastoma - and get one step closer to finding a cure.*"

ENDS

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