

Childhood dementia: the collective impact and therapeutic landscape

childhood
dementia
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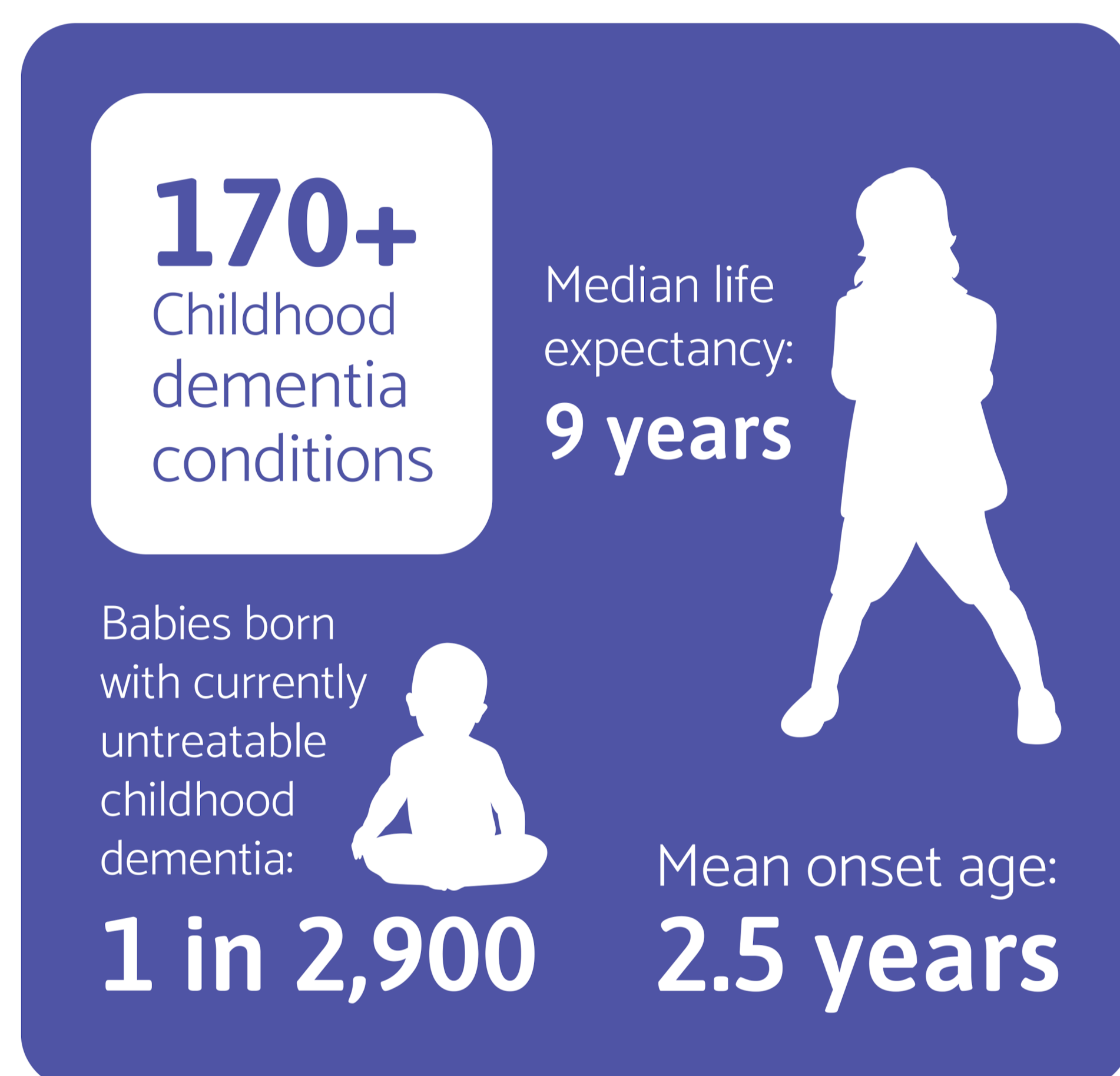
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What is childhood dementia?

Childhood dementia is a devastating group of disorders with a high level of unmet need. Typically monogenic in origin, this collective of individual neurodegenerative conditions are defined by a progressive impairment of neurocognitive function, presenting in childhood and adolescence.

In contrast to adult-onset dementia, childhood dementia has received little recognition in the medical literature and the lay media. This is due to the individual rare and ultra-rare disorders being considered individually based on their pathology rather than as a broader clinical phenotype as the adult dementias are.



We undertook a systematic review and burden of illness study to understand the spectrum of childhood dementia disorders and estimate the collective incidence, prevalence and life expectancy. This study identified 170 individual genetic conditions, caused by mutations in at least 200 genes, plus an additional multitude of genetic causes of mitochondrial disease. The largest proportion of births belongs to a group of conditions designated as 'diseases not otherwise categorised' (27%) and includes disparate disorders such as Rett syndrome and juvenile Huntington disease. This was followed by the lysosomal disease (22%) and mitochondrial disorder (20%) categories.

The Childhood Dementia Knowledgebase

The Childhood Dementia Knowledgebase contains key statistics for each of the childhood dementia disorders. This includes:

- Incidence
- Prevalence
- Life expectancy
- Age of onset and diagnosis
- Genetic cause
- Signs and symptoms
- Disease mechanisms (coming soon)

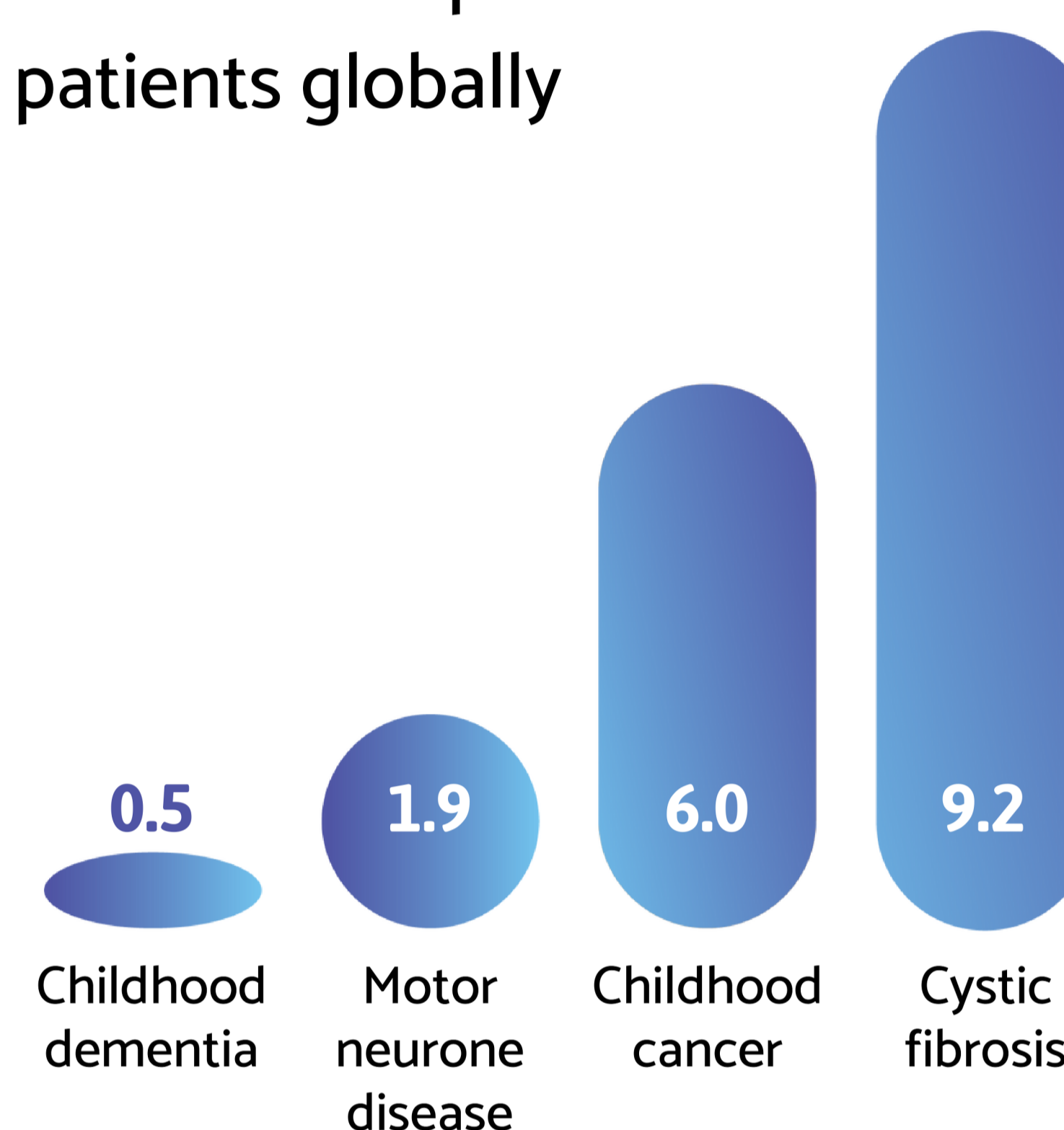
It provides data on the collective impact of childhood dementia that can be used for advocacy and research funding applications. This powerful resource gives the ability to identify disorders with a particular symptom or which are amenable to a certain treatment approach, encouraging the concurrent study of multiple childhood dementia disorders.

References

Elvidge et al. Brain. (in press)
Childhood Dementia Initiative. State of Childhood Dementia 2022 (2022)

The clinical trial landscape

Clinical trials per 1000 patients globally



Analysis of the landscape of childhood dementia research globally revealed just 353 childhood dementia clinical trials have been undertaken across 48 countries since 1990. The inadequacy of this clinical trial activity is especially striking in relation to other better-known disease groups.

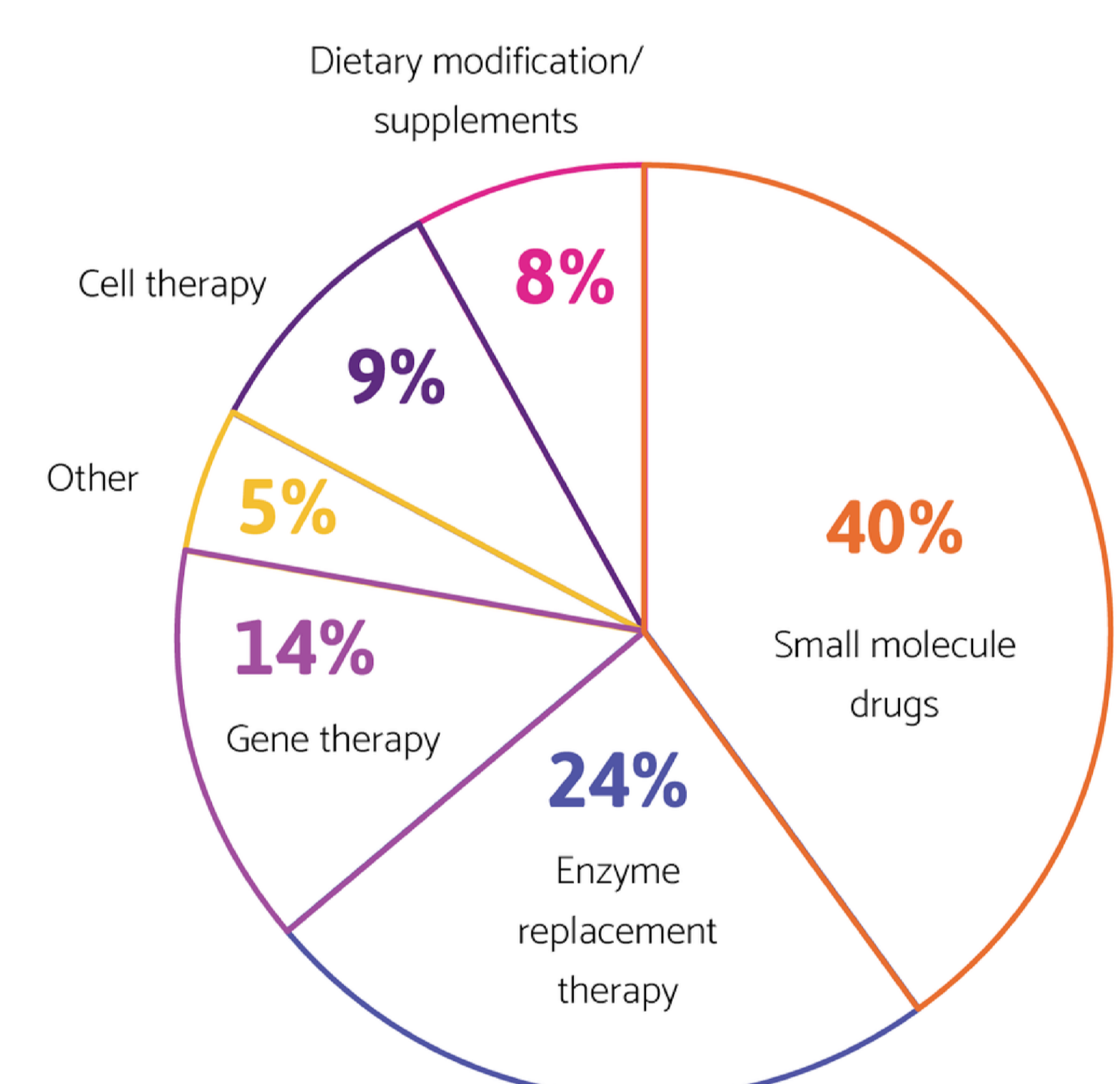
The number of childhood dementia clinical trials per patient was 18 times less than cystic fibrosis, 12 times less than childhood cancer and 4 times less than motor neurone disease (MND).

In addition, the clinical trials that are occurring for childhood dementia are out of step with need. Some relatively common disorders have little or no clinical trial activity and more than half of the childhood dementia disorders currently have no clinical trial options available to patients anywhere in the world. It is hoped that the collective consideration of the childhood dementia disorders will address this inequity and increase research activity across the spectrum of disorders.

Types of potential treatments tested in clinical trials

Encouragingly, gene therapies make up a sizable proportion of clinical trials. This is increasing every year and signals progress towards cutting-edge treatments and cures that target the root causes of childhood dementia.

The highest proportion of trials focused on testing small molecule drugs (40%). These drugs may not all be curative but could significantly slow progression and improve quality of life, especially if administered in combination. Many of these drugs could be widely applicable across multiple childhood dementia disorders with common attributes. However, they have only ever been tested in clinical trials one disorder at a time. This means that opportunities are being missed for more patients to benefit from potential treatments.

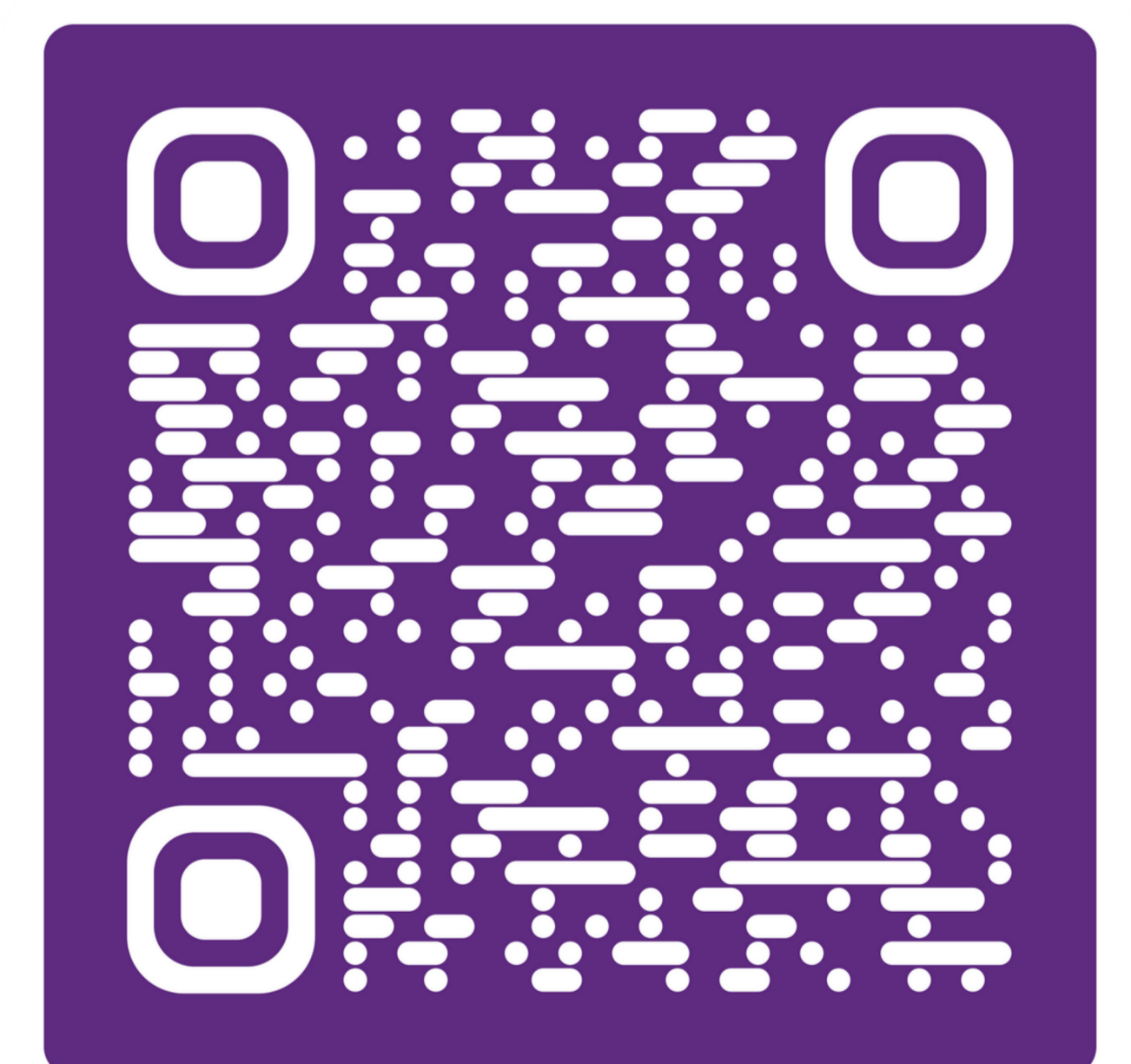


Conclusion

For most children with dementia, there are no treatments available beyond symptom management. The burden of childhood dementia on those who care for them is immense and families' needs are not currently met by health and social systems.

Collectively addressing childhood dementia is a world-first approach. It provides opportunities for greater scale, impact and improvement of policy, services and therapy development. It represents a paradigm shift in how these children are viewed, cared for and treated.

Childhood Dementia Initiative is bringing together the world's brilliant health and medical researchers to work collaboratively to develop new effective treatments. To stay up to date on the latest news and opportunities, to help us identify research priorities and increase opportunities for collaboration, join the growing Childhood Dementia Research Alliance now.



For a PDF of this poster, to view the Childhood Dementia Knowledgebase, and to join the Childhood Dementia Alliance, scan the QR code or visit: childhooddementia.org/for-researchers

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