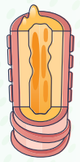
Early Intervention in Cystic Fibrosis: Limiting Multisystem Disease Progression

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EMJ European Medical Journal

The burden of CF



CF is a progressive autosomal recessive disease caused by variants in the *CFTR* gene,[1-3] characterised by the accumulation of sticky mucus in multiple organs, of which the lungs, GI tract and pancreatic ducts are the most commonly affected.[4]



CF is an inherited disorder[1,5,6] associated with high symptom burden and reduced quality of life.[7,8]



Structural lung disease is present within the first year of life, contributing to associated morbidity and mortality rates.[9-12] 85% of infants with CF have exocrine pancreatic insufficiency.[13]

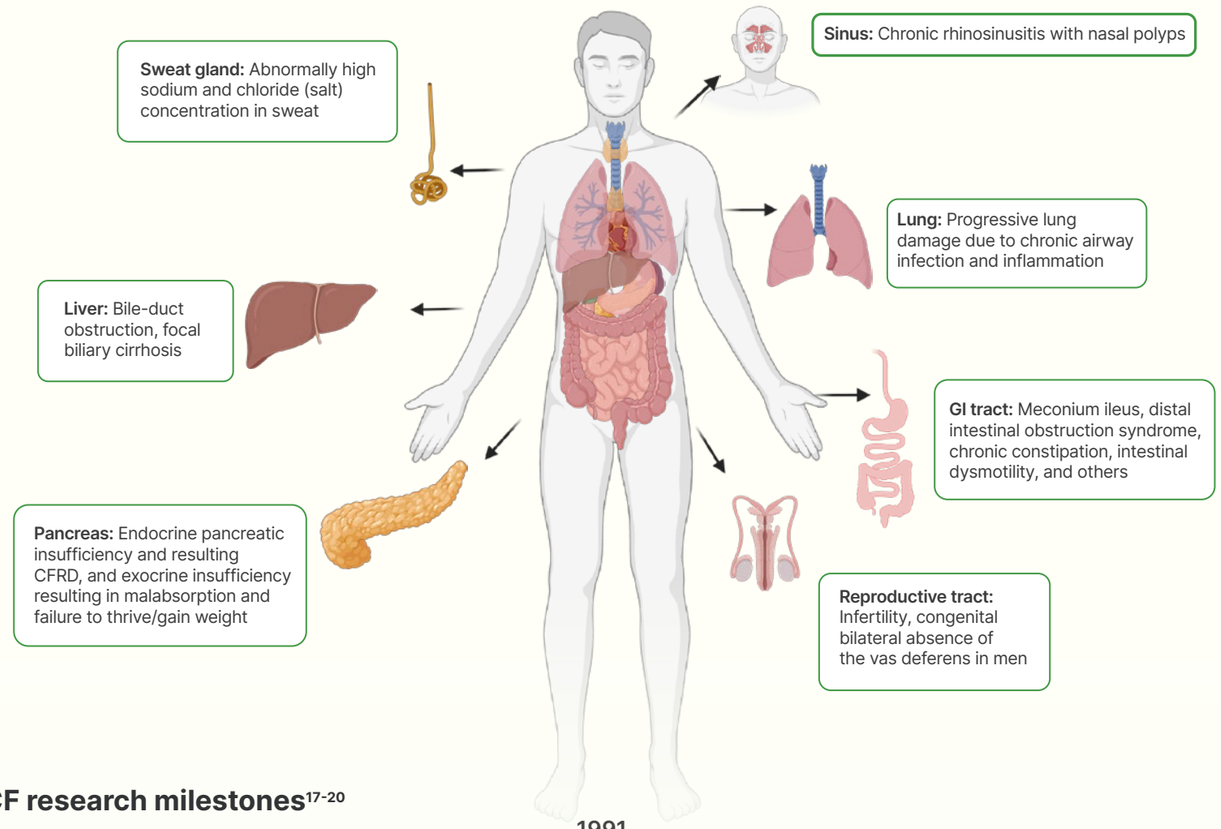


In countries with CF screening programmes such as the USA, CF is typically diagnosed shortly after birth, enabling early specialist care and timely intervention.[14]



While CF has traditionally been thought of as a lung disease, it affects various other organ systems, including the pancreas, sweat glands, liver, and GI tract, extending into adulthood.[1-3,15]

CF is a multisystem disease that impacts multiple organs[1-3,16]



Early intervention can change the trajectory of CF[6]



May reduce progression of irreversible damage



May support healthy growth and development



May reduce cumulative disease burden

Why intervene early?

- Early treatment of CF is crucial to improving long-term health outcomes and potentially improving the disease trajectory.[26-30]
- Early treatment initiation may delay, reduce, or limit progression of organ damage, reduce cumulative disease burden, and potentially support healthy growth.[26-30]
- Despite advances in CF therapy and care, unmet needs still remain for some patients living with CF.

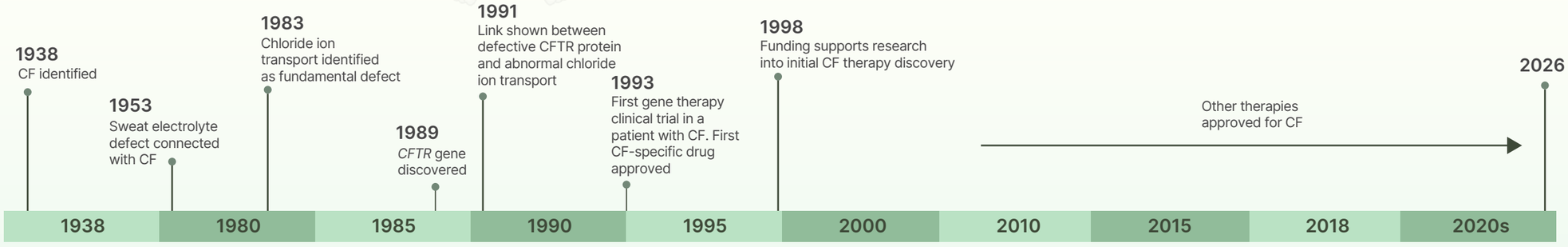
KEY MESSAGES

CF is a systemic disease that requires a whole-body, multidisciplinary approach.[1-3]

CF begins before symptoms become apparent. Disease-related changes in the lungs, pancreas, and GI tract can start early in life, making timely diagnosis and intervention critical.[6,9,13]

Significant improvements in care have changed the outlook of CF; however, unmet needs still remain.

CF research milestones[17-20]



Timeline illustrating major advances in the understanding and treatment of CF.

Patient outlook then

In the 1980s, most people with CF did not reach adulthood.[2,21]



1980s: A life expectancy of <20 years

Significant improvements in care have changed the outlook of CF.[25]

Patient outlook now

The median age of survival now is above 50 years in Canada, Australia, New Zealand, France, and the USA[2,21-23] with latest estimates reaching 65 years and beyond.[24]



2026: Living into their 50s and beyond

Abbreviations

CF: cystic fibrosis; CFRD: cystic fibrosis-related diabetes; CFTR: cystic fibrosis transmembrane conductance regulator; GI: gastrointestinal.

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