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Progress in cardiac amyloidosis

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Systemic amyloidoses are a group of rare diseases caused by deposition of protein fibrils. This talk will focus on major advances in the approaches to diagnosis, changing epidemiology and recent advances in treatment.Light chain (AL) amyloidosis remains the most frequently identified type but cardiac transthyretin amyloidosis is being increasingly recognised. Senile cardiac amyloidosis appears to be an epidemic awaiting diagnosis. Mass spectrometry using laser capture microdissection of a tiny amount of amyloid deposits from histological sections has enabled improved amyloid fibril typing. Understanding of proteotoxicity of amyloidogenic precursors has paved the way for new therapeutic approaches. Developments in cardiac magnetic resonance imaging such an Eq-CMR and T-1 mapping has led to accurate quantitation of the myocardial interstitial deposits for diagnosis and response assessment. ^{99}mTc-DPD/PyP scintigraphy is transforming evaluation of cardiac amyloidosis. The availability of novel chemotherapy agents and better selection of patients for autologous stem cell transplantation have enabled the delivery of therapy in AL with less toxicity and improved outcomes. An array of novel agents, including RNA inhibitors, stabilisers of amyloid precursor proteins, inhibitors of fibril formation and immunotherapeutic targeting of amyloid deposits are all now in clinical development offering great hope for specific and effective new therapies.

Biography

Ashutosh Wechalekar is a Reader in Medicine at University College London Medical School and the Royal Free London NHS Foundation Trust. He trained in medicine at Medical College Nagpur, India and when on to do further training in haematology in the UK and Canada. He joined the UK National Amyloidosis Centre funded in 2004 and is now a senior faculty at the centre. His scientific interests are focus on biomarkers in cardiac amyloidosis, novel imaging methods for the heart in all types of amyloidosis (especially DPD scintigraphy – he now has the largest cohort in the world of over 700 patients with amyloidosis imaged by this modality), characterization and treatment of systemic AL amyloidosis with a focus on the study new and novel agents in these disorders. He has published extensively in these areas.

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