

Highlights from this issue

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BEYOND THE 'HOW ARE YOU, DEAR'

We all want the patient to tell us how they feel, but assessing quality of life has gone way beyond this. Quality of life questionnaires are increasingly important clinical trial end-points, and, in cystic fibrosis in particular, where FEV₁ is often too stable to be useful to be used as a clinical trial end-point, the use of questionnaires is assuming increasing prominence. In this issue, Abbott *et al* report longitudinal data on Health-related quality of life (HRQoL) in cystic fibrosis over a 12 year period and correlated this with clinical outcomes. Surprisingly, both FEV₁ and HRQoL declined slowly, something that was not predicted by cross-sectional studies. Optimism was a good thing (despite the gloomy Nero Wolfe dictat 'The Pessimist only gets pleasant surprises, the Optimist only unpleasant'). Of great importance was that some (presumably optimistic) reported no change in HRQoL while their lung function declined. For us, this manuscript emphasises the importance of really long-term longitudinal studies if relationships between variables is to be understood (an early bidder for the gold medal in 2013), and that FEV₁ and HRQoL are complementary (*see page 149*).

THE FDA AND LONG ACTING B₂-AGONIST (LABA) SAFETY

One positive outcome of the global financial crisis might be the focusing of our efforts on what is clearly important and cost effective. Not so at the Food and Drug Administration who have mandated a series of large clinical trials of LABA/inhaled corticosteroid safety at a total cost of many millions of dollars. Sears (*see page 195; Hot Topic*) points out that these trials can only answer a question for which there is already a clear answer and do not have a hope of identifying a mortality difference. These studies are therefore a huge waste of valuable resources which could be profitably spent elsewhere. Another own goal by the FDA, an organisation that must be a serious contender for our annual foot in mouth award, leaving even NICE and the UK Department of Health trailing in their wake. Beasley and colleagues (*see page 119*) suggest that a easy, quick and cost effective win would be to apply a universal

ban on LABA monotherapy, a practice which all accept is dangerous in asthma yet, according to Morales *et al* (*see page 192*), remains depressingly widespread.

PRIMARY CILIARY DYSKINESIA

The structure strikes back! Primary ciliary dyskinesia (PCD) is often harder to think of than to diagnose. However, when thought of the diagnosis may still be difficult. Last year the geneticists staked a claim for the usefulness of gene analysis, leading to the sparks flying as those who pushed for a functional definition of the disease joined battle with them. In this issue of *Thorax*, the morphologists join the fray. Standard transmission electron microscopy often fails to reveal abnormalities in ciliary structure in cases diagnosed with PCD on unequivocal clinical, functional and genetic grounds. Now sophisticated electron microscopy (EM) tomographic techniques are revealing abnormalities in some of these cases (*see page 190*). However, for the moment peace has broken out; this technique was able to demonstrate that projections associated with the central pair apparatus were associated with mutations in the *hyd1n* gene, helping to validate this mutation as disease-causing. EM tomography is the first really major advance in ciliary electron microscopy in recent years, and undoubtedly will shed more light on ciliary structure in health and disease.

SEPTIN AND IDIOPATHIC PULMONARY FIBROSIS (IPF): A NEW USE FOR AN OLD DRUG?

Editors, reviewers and editorialists (*see page 123*) were stunned by the

findings of Shulgina *et al* (*see page 155; Editors' Choice*) that treatment with old fashioned Septrin reduced mortality from IPF by as much as five times. There are important caveats: mortality was not the primary outcome; the findings in the intention to treat analysis were much less impressive as treatment was not always well tolerated; and the benefits were much more obvious in the patients who were receiving immunosuppressive treatment, a practice that is now defunct. Even so, the findings are striking and are surely a strong basis for a second larger trial, at least when the IPF doctors can decide on what they should use as outcomes! This study was born out of old fashioned clinical observation by a busy clinician with a special interest in the field. We congratulate Dr Varney, the TIPAC study team and the NIHR research for patient benefit programme for developing the concept and making the trial happen. We await the results of 'TIPAC-2' with great interest.

NOT THE BLEEDINGLY OBVIOUS

This 41-year-old with known myelofibrosis became breathless and wheezy over a 12-month period. A case for tiotropium or another expensive inhaler, or a different approach needed? The CT scan is shown in the figure and the pathology is our cover illustration: what's going on, what do we do next and what's the diagnosis? No cheating, answers on *page 199*.

