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7

**OPTIMISING PREVENTION OF
PNEUMOCOCCAL DISEASE WITH
PNEUMOCOCCAL CONJUGATE VACCINES
PFIZER SYMPOSIUM, 23 OCTOBER 2010,
13:00-14:00**

N. Stephens

ESAT Vaccines, Pfizer, Paris, France

Paediatricians play a critical role in the battle against pneumococcal diseases, with a front-line position in its ongoing surveillance and prevention. The addition of pneumococcal conjugate vaccines (PCVs) to the armamentarium has dramatically changed the landscape in this battle; PCV7 was introduced almost a decade ago, and has been shown to have had a strong impact on all pneumococcal diseases caused by vaccine serotypes.

This symposium will open by looking at the burden of invasive and non-invasive pneumococcal disease across Europe, comparing the incidence and prevalence pre- and post-PCV7, and examining the impact of the different vaccination schedules in force.

Putting all this into practice, a member of our distinguished faculty will present an interactive case study on paediatric empyema, giving the audience the chance to vote and share their views on treatment strategies for invasive pneumococcal

diseases.

Finally, we will turn to consider the remaining burden of pneumococcal disease, looking at the broader coverage of the 13-valent PCV formulation, and the practicalities of transitioning from PCV7, as well as the opportunities for providing broader protection to already fully vaccinated children.

We very much hope you will join us for what promises to be a lively and educational event as we consider how best to optimise the prevention of pneumococcal diseases with PCVs.

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**TREATMENT OPTIONS FOR NP-C: CHANGING
OUR APPROACH TO THE DISEASE**

M. Patterson

Division of Child and Adolescent Neurology, Mayo Clinic, Rochester, MN, USA

Definitive disease-modifying therapy for Niemann-Pick disease, type C (NPC) would either correct the primary genetic defect or replace the deficient gene product [1]. Neither strategy is currently applicable to patients with *NPC1* mutations, but replacement of the NPC2 protein by haematopoietic stem cell transplantation is theoretically possible, although proof of principle in humans is lacking.

Alternative approaches involve interdicting pathways downstream to the primary mutation that lead to cellular dysfunction. These approaches include reduction of substrate burden, replacement of deficient downstream products, suppression of inflammation, inhibition of apoptosis and activation of alternate pathways. Of these approaches, only substrate reduction therapy has been studied and approved for use in humans.

Miglustat is an iminosugar that competitively inhibits glucosylceramide synthase, the enzyme catalysing the rate-limiting step in the synthesis of higher order glycosphingolipids. Miglustat has been shown to delay the onset of symptoms in the NPC murine model. Both prospective and retrospective human studies have also found evidence of a beneficial effect on disease progression in NPC [2-5]. The availability of such a treatment mandates early diagnosis of patients with NPC, since those without irreversible disease will be likely to benefit most. Currently, there is no readily available test for NP-C, but in future, oxysterols or other biomarkers may facilitate screening for NPC.