

PAYER PERSPECTIVES ON THE REQUIREMENTS FOR A NOVEL INHALED COMBINATION TO IMPROVE MANAGEMENT OF *PSEUDOMONAS AERUGINOSA* INFECTIONS IN CYSTIC FIBROSIS PATIENTS – WHAT IS NEEDED?

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INTRODUCTION

The spread of antimicrobial resistance (AMR) is increasingly recognised as both a healthcare and an economic threat and there is consequently an urgent need to develop new and effective measures against bacterial infections. Cystic fibrosis (CF), with its propensity for sufferers to develop ultimately lethal chronic bacterially-caused respiratory infections, is one of the disease areas at greatest risk in this regard. With last resort antibiotics routinely prescribed prophylactically to manage these infections, further development of resistance to the effects of these antibiotics would drastically limit intervention possibilities for people with CF. A suitable and effective alternative intervention for these persistent bacterially-caused respiratory infections is therefore needed.

NX-AS-401 is an inhaled treatment with a novel mechanism of action that has potential to reduce the required dose of Standard of Care antibiotics that it is used alongside to counter chronic *Pseudomonas aeruginosa* lung infections in CF.

RESULTS

The burden of chronic *P. aeruginosa* lung infection in CF is recognised as being high, and having a significant downstream cost. With up to 80% of hospital admissions for adults with CF still being related to *P. aeruginosa* exacerbations, payers highlighted a significant unmet need for effective treatments that will improve the long term management of chronic *P. aeruginosa* infections.

The most important need was around maintaining or improving lung function and reducing exacerbations and subsequent hospitalisations. Ease of use in a chronic setting was also highlighted, as was any opportunity to reduce exposure to aminoglycosides (Fig. 1). This mirrors cost effectiveness model findings presented at ISPOR's 22nd Annual International Meeting² and raises the question of how AMR can best be valued (Fig. 2 and 3).

OBJECTIVES & METHODS

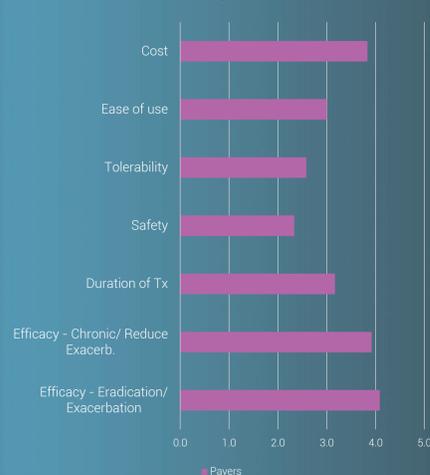
Around 45% of CF patients are chronically infected with *P. aeruginosa* which is the primary cause of pulmonary deterioration and ultimately mortality¹. Most of the Standard of Care antibiotics are available at low cost, raising the question of how an innovative antimicrobial would be valued. The objective of this research was therefore to understand payers' perspectives of unmet needs, value drivers and evidence requirements around this novel treatment as well as exploring their willingness to pay for such an adjunctive treatment.

Forty qualitative telephone Interviews were conducted with payers and physicians across the EU5 and USA, exploring perceptions of unmet need, value drivers (scored on a 1-5 analogue scale), response to the TPP, willingness to pay for an additional product, key evidence requirements and optimum positioning of an intervention with a novel mechanism of action.

FIG. 1 - PERCEPTION OF UNMET CLINICAL NEED

New and effective treatments to improve the long term management of chronic *P. aeruginosa* infections were identified as the main area of unmet clinical need.

Respondents rated unmet needs on a scale of 1 – 5 where 5 is highest unmet need



"(I am) not satisfied with current antibiotic treatment options in chronic *Pseudomonas* patients; advanced colonisation in these patients is not controlled with the current antibiotics, leading to exacerbations"

"(It) remains a big problem... Exacerbations take significant resources. It is ultimately long term treatment and exacerbations that cost"

"*Pseudomonas* is the most frequent bacterial association with deterioration in CF patient"

"(This is a) Really important area. There's been lot's of political interest in (the) last few years. With the pipeline dried up and nothing new for 20 years, it is quite worrying. In the context of CF, AB use is extreme and resistance is high."

FIG. 2 – VALUE DRIVERS

Key value drivers reported by payers for an alternative inhaled adjunct to current Standard of Care for chronic bacterial respiratory infections in CF (from L to R in descending order of priority).

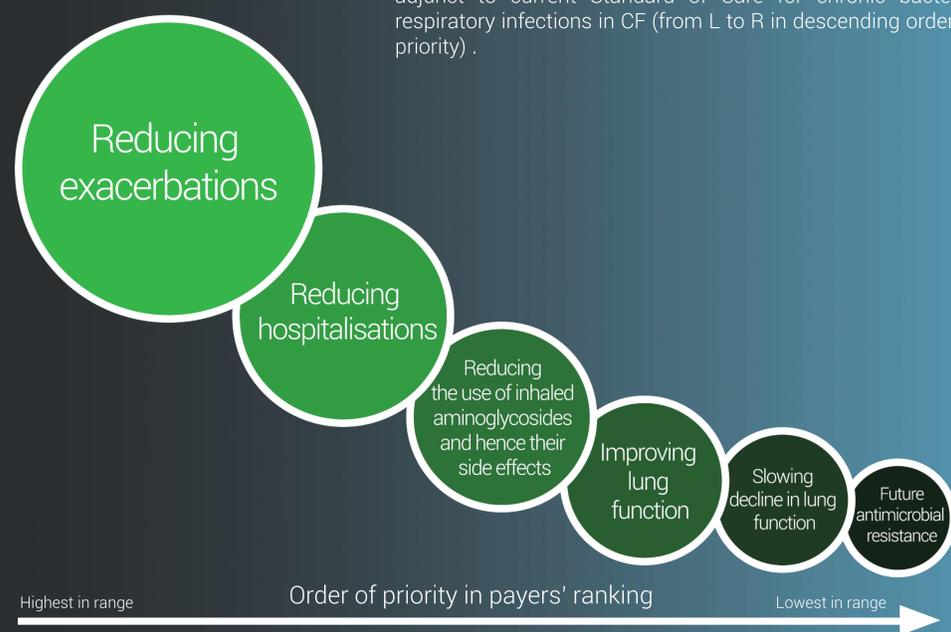
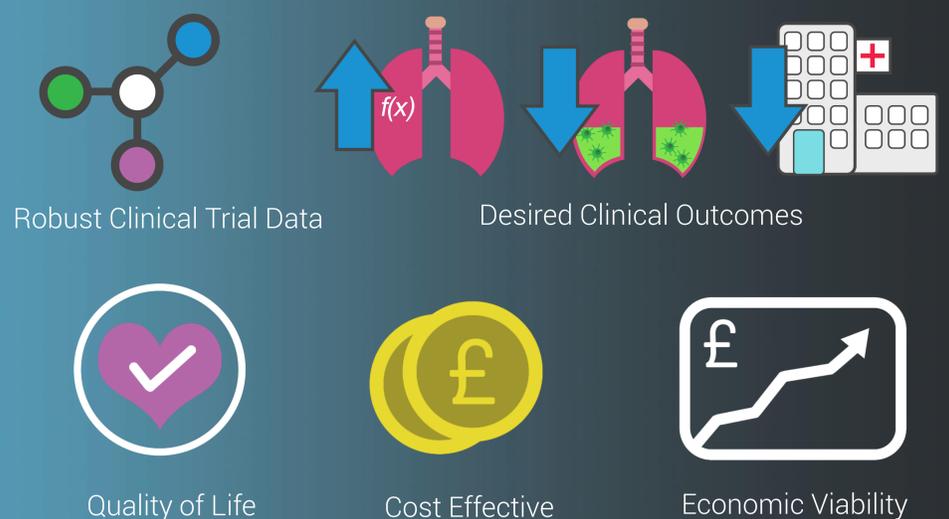


FIG 3. – REQUIRED EVIDENCE FOR REIMBURSEMENT

Respondents were unanimous in their view that any new drug's target product profile would need to be based on a substantial evidence base if it is to be reimbursable at prices similar to current inhaled comparators such as inhaled Tobramycin.



CONCLUSIONS

A significant need exists for additional treatments to improve the long term management of chronic *P. aeruginosa* infections in patients with CF. Treatments targeting this comorbidity should focus on a reduction in exacerbations, hospitalisations, AMR development and concomitant drug use if they are to meet this clinical unmet need in a cost effective and clinically relevant manner.

The profile of NX-AS-401 with its potential to deliver desired clinical outcomes whilst simultaneously reducing aminoglycoside use and AMR development is a pioneering approach to how AMR can be valued in a proactive manner and hence could provide a valuable contribution to the antimicrobial stewardship agenda and ultimately the long term health of people with CF.

REFERENCES

- 2016 Cystic Fibrosis Foundation Patient Registry Highlights Bethesda, Maryland ©2017 Cystic Fibrosis Foundation.
- Graz, M., et al. 2017. *Exploring Economic Value in Treating Pseudomonas Aeruginosa Infections in Cystic Fibrosis – Early Modelling to Inform Development*. ISPOR 22nd Annual International Meeting, Boston, USA.