

#### Clinical trial results: Trial of Optimal Therapy for Pseudomonas Eradication in Cystic Fibrosis Summary

Results information	

#### **Trial information**

**Trial identification** 

#### Additional study identifiers

Sponsors	

Paediatric regulatory details	

Results analysis stage	

General information about the trial

Population of trial subjects	
Subjects enrolled per country	

Subjects enrolled per age group	

#### Recruitment

**Pre-assignment** 

Period 1

#### Arms

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Arm title	

Arm title		
	Arm title	

Number of subjects in period 1	

#### Period 2

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Arm title	

Arm title	

Number of subjects in period 2	

# Reporting groups

Reporting group values		

End points reporting groups		

Primary: Primary efficacy assessment - Successful eradication of P. aeruginosa three months after the start of treatment, remaining infection free through to 15 months after the start of treatment

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End point values		

Statistical analysis title	

## Primary: Primary efficacy assessment - Successful eradication of P. aeruginosa three months after the start of treatment, remaining infection free through to 15 months after the start of treatment

	Image: Constraint of the second sec

Statistical analysis title	

### Primary: Primary efficacy assessment – Sensitivity analysis 6: no T15 window (Post hoc)

End point values		

Statistical analysis title	

Secondary: Time to reoccurrence of original P.aeruginosa infection - unknown strains assumed to be same as baseline			


End point values		

Statistical analysis title	
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strains assumed to be different to baseline		

End point values		

Statistical analysis title	

#### Secondary: Re-infection with a different strain of P.aeruginosa

End point values		

Statistical analysis title	
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Secondary: Post hoc analysis – Genotyping results		

End point values		

Secondary: Lung function - FEV1% predicted		

End point values		

Statistical analysis title	

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Statistical analysis title	

Secondary: Lung function - FVC% predicted		

End point values		

Statistical analyses	
Statistical analysis title	

Statistical analysis title	
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Statistical analysis title	
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Statistical analysis title	

Secondary: Lung function - FEF25-75% predicted		

End point values		

Statistical analysis title	

Statistical analysis title	
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Statistical analysis title	
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Secondary: Oxygen saturation		

End point values		

Statistical analysis title	

Statistical analysis title	
Statistical analysis title	

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Statistical analysis title	

Secondary: Growth and nutritional status - Height z-scores		

End point values		

Statistical analyses	
Statistical analysis title	

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Statistical analysis title	
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Statistical analysis title	

Secondary: Growth and nutritional status - Weight z-scores

End point values		

Statistical analysis title	

Statistical analysis title	
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Statistical analysis title	
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#### Secondary: Growth and nutritional status - BMI z-scores

End point values		

Statistical analysis title	

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Statistical analysis title	

Secondary: Growth and nutritional status - BMI			

End point values		

Statistical analyses	
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Statistical analysis title	

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Secondary: Number of pulmonary exacerbations - median number of exacerbations during the 15 months following treatment commencement

End point values		

Statistical analysis title	
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Secondary: Number of pulmonary exacerbations - Number of participants experiencing at least one exacerbation during the first 15 months of follow up		
End point values		
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Statistical analysis title	

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Secondary: Admission to hospital - Number of participants experiencing at least o hospital stay during the first 3 months of treatment	ne

Statistical analyses	
Statistical analysis title	

Secondary: Admission to hospital - Number of participants experiencing at least one
hospital stay during the 12 months following treatment

End point values		

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Statistical analysis title	

Secondary: Admission to hospital - Number of participants experiencing at least one hospital stay between 15 and 24 months			

End point values		

Statistical analysis title	

# Secondary: Number of days spent as an inpatient in hospital - During treatment phase

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End point values			

#### Statistical analyses

Statistical analysis title	

## Secondary: Number of days spent as an inpatient in hospital - During the 12 months post treatment phase

End point values		

Statistical analysis title	

Secondary: Number of days spent as an inpatient in hospital - between 15 and 24 nonths follow up

End point values		

Statistical analysis title	

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End point values		

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Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Vitality – Self Report		

End point values		

Statistical analysis title	
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Statistical analysis title	

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Secondary: CFQ - Emotional functioning – Self Report		

End point values		

Statistical analysis title	

Statistical analysis title	

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Secondary: CFQ - Social functioning – Self Report		

End point values		

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Secondary: CFQ - Body Image – Self Report		

Statistical analysis title	

Statistical analysis title	

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Secondary: CFQ - Eating Problems – Self Report		

End point values		

Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Treatment Burden – Self Report		

End point values		

Statistical analyses	
Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Health Perceptions – Self Report

End point values		

Statistical analysis title

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Secondary: CFQ - Weight – Self Report		

End point values		

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Secondary: CFQ - Respiratory Symptoms – Self Report		

End point values		

Statistical analyses	
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Statistical analysis title	

Secondary: CFQ - Digestive Symptoms – Self Report

End point values		

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End point values		

Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Role/school functioning – Parent/Carer		

End point values		
Statistical analyses		
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Statistical analysis title		

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Statistical analysis title	

Secondary: CFQ - Vitality- Parent/Carer		

End point values		

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### Secondary: CFQ - Emotional functioning – Parent/Carer

End point values		

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Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Body Image – Parent/Carer	

End point values		

Statistical analyses	
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Statistical analysis title	

Secondary: CFQ - Eating Problems- Parent/Carer

End point values		

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End point values		

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End point values		

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Statistical analysis title	

Secondary: CFQ - Weight - Paren	nt/Carer

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End point values		

Statistical analysis title

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### Secondary: CFQ - Respiratory Symptoms- Parent/Carer

End point values		

Statistical analysis title	

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Statistical analysis title	

Secondary: CFQ - Digestive symptoms - Parent/Carer	

End point values		

Statistical analyses	
Statistical analysis title	

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Statistical analysis title	

### Secondary: Number of patients with at least one positive result of MRSA by 3 months

End point values		

Secondary: Number of patients with at least one positive result of Burkholderia cepacia complex (BC) by 3 months		

End point values		

#### **Statistical analyses**

Secondary: Number of patients with at least one positive result of Candida by 3

months	

End point values		

Statistical analysis title	

### Secondary: Number of patients with at least one positive result of Aspergillus by 3 months

End point values		

Statistical analysis title	
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Secondary: Number of patients with at least one positive result of MRSA by 15 months

End point values		

### Secondary: Number of patients with at least one positive result of Burkholderia cepacia complex (BC) by 15 months

End point values		

Statistical analyses	
Statistical analysis title	

Secondary: Number of patients with at least one positive result of Candida by 15 months

End point values		

Statistical analysis title	

Secondary: Number of patients with at least one positive result of Aspergillus by 15 months

End point values		

# Secondary: Number of patients with at least one positive result of MRSA by 24 months

End point values		

## Secondary: Number of patients with at least one positive result of Burkholderia cepacia complex (BC) by 24 months

End point values		

Statistical analysis title	
Statistical analysis title	

Secondary: Number of patients with at least one positive result of Candida by 24 months

End point values		

Statistical analysis title	

Secondary: Number of patients with at least one positive result of Aspergillus by 24 months

End point values		

Statistical analysis title	
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# Secondary: Carer Burden - Median number of days absence during the 15 months following randomisation

End point values		

Statistical analysis title	

Secondary: Carer Burden - Number of carers experiencing at least one episode of absence during the first 15 months of follow up		

End point values		

Statistical analysis title	
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Secondary: Participant burden - Median number of days absence during the 15	
months following treatment randomisation	

End point values		

Statistical analysis title	
Statistical analysis title	

Secondary: Participant burden - Number of participants experiencing at least one episode of absence during the first 15 months of follow up		
End point values		
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Statistical analysis title	
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Secondary: Number of days spent as an inpatient in hospital during treatment phase - Sensitivity Analysis 1

End point values		

Statistical analyses	
Statistical analysis title	

Secondary: Number of days spent as an inpatient in hospital during 12 months post treatment- Sensitivity Analysis 1			

End point values		

Statistical analysis title	
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Secondary: Number of days spent as an inpatient in hospital between 15 and 24 months - Sensitivity Analysis 1					

End point values		

Statistical analysis title	

Secondary: Number of days spent as an inpatient in hospital during treatment phase

- Sensitivity Analysis 2	

End point values		

Statistical analysis title	

Secondary: Number of days spent as an inpatient in hospital during 12 months post treatment- Sensitivity Analysis 2		

End point values		

Statistical analysis title	
Statistical analysis title	

### Secondary: Number of days spent as an inpatient between 15 and 24 months-Sensitivity Analysis 2

End point values			

## Secondary: Number of patients with at least one positive result of another organism during the study

End point values		

## Other pre-specified: Primary efficacy assessment – Sensitivity analysis 1: All patients followed up past 3 months but with no 15 month sample classified as successes



End point values		

# Other pre-specified: Primary efficacy assessment – Sensitivity analysis 2 – All patients followed up past 3 months but with no 15-month sample classified as failures

End point values		

Statistical analysis title	

# Other pre-specified: Sensitivity analysis 3 – All patients followed up for more than 15 months but with no 15-month sample classified as success/failure in accordance with the next sample taken after the 15-month window

End point values		

Statistical analysis title	

Other pre-specified: Primary efficacy assessment – Sensitivity analysis 4: Centre effect		

End point values		

Other pre-specified: Primary efficacy assessment – Sensitivity analysis 5: T3 and T15 extended to +10 weeks (Post hoc) - successful eradication			

End point values		

### Statistical analyses

Statistical analysis title

### Other pre-specified: Primary efficacy assessment – Sensitivity analysis 5: T3 and T15 extended to +10 weeks (Post hoc) - unsuccessful eradication

End point values		

# Other pre-specified: Sensitivity analysis: Time to reoccurrence of original P.aeruginosa infection (Those with reoccurrence but unknown strain assumed to be same as baseline and TO adjusted to be date of treatment commencement rather than date of randomisation)

End point values		

Statistical analysis title	

# Other pre-specified: Sensitivity analysis: Time to reoccurrence of original P.aeruginosa infection (Those with reoccurrence but unknown strain assumed to be different to baseline and T0 adjusted to be date of treatment commencement rather than date of randomisation)

End point values		

Statistical analysis title	

### Other pre-specified: Additional analysis 1: Time to first pulmonary exacerbation

End point values		

Statistical analysis title	

## Other pre-specified: Additional analysis 2: At least one exacerbation during the treatment phase

End point values		

Statistical analysis title	
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### Other pre-specified: Additional analysis 2: At least one exacerbation during the first year following treatment

End point values		

Other pre-specified: Additional analysis 2: At least one exacerbation during the	
second year following treatment	

End point values		

Statistical analysis title	
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Other pre-specified: Sensitivity analysis - Number of participants experiencing at least one hospital stay during the 12 months following treatment		

End point values		

Statistical analysis title		

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Other pre-specified: Sensitivity analysis - Admission to hospital - Number of participants experiencing at least one hospital stay between 15 months and 24 months

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End point values		

### Adverse events information

Dictionary used	
Reporting groups	

Serious adverse events		




Non-serious adverse events		



### More information

### Substantial protocol amendments (globally)

Date	Amendment
Interruptions (globally)

Limitations and caveats