# Clinical trial results:
# A Phase II, randomized, double-blind, placebo-controlled, multi-center study to evaluate the efficacy, safety, tolerability and pharmacokinetics of orally administered combination of GLPG3067, GLPG2222 and GLPG2737, in adult subjects with cystic fibrosis homozygous or heterozygous for F508del CFTR.

## Summary

| | |
|---|---|
| EudraCT number | 2018-000098-61 |
| Trial protocol | GB |
| Global end of trial date | 17 July 2018 |

**Results information**

| | |
|---|---|
| Result version number | v1 (current) |
| This version publication date | 16 October 2020 |
| First version publication date | 16 October 2020 |
| Summary attachment (see zip file) | Cancelled before enrollment statement (glpg3067-cl-202-synopsis.pdf) |

## Trial information

**Trial identification**

| | |
|---|---|
| Sponsor protocol code | GLPG3067-CL-202 |

**Additional study identifiers**

| | |
|---|---|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | - |
| WHO universal trial number (UTN) | - |

Notes:

**Sponsors**

| | |
|---|---|
| Sponsor organisation name | Galapagos NV |
| Sponsor organisation address | Generaal De Wittelaan L11 A3, Mechelen, Belgium, 2800 |
| Public contact | Medical Information, Galapagos NV, +32 15342 900, medicalinfo@glpg.com |
| Scientific contact | Clinical Trial Information Desk, Galapagos NV, +32 15342 900, rd@glpg.com |

Notes:

**Paediatric regulatory details**

| | |
|---|---|
| Is trial part of an agreed paediatric investigation plan (PIP) | No |
| Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial? | No |
| Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial? | No |

Notes:

## Results analysis stage

| | |
|---|---|
| Analysis stage | Final |
| Date of interim/final analysis | 17 July 2018 |
| Is this the analysis of the primary completion data? | No |

| | |
|---|---|
| Global end of trial reached? | Yes |
| Global end of trial date | 17 July 2018 |
| Was the trial ended prematurely? | No |

Notes:

## General information about the trial

Main objective of the trial:

To assess changes in sweat chloride concentration as a biomarker of CFTR ion channel function for dual combination (GLPG3067 and GLPG2222) and triple combination (GLPG3067, GLPG2222, and GLPG2737) treatment, in adult CF subjects who are homozygous for CFTR mutation F508del, and in adult CF subjects who are heterozygous for CFTR mutation F508del (with a mutation on the second allele, which is non-responsive to potentiator).

Protection of trial subjects:

This clinical study will be conducted in accordance with the current International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use - Good Clinical Practice (ICH-GCP) Guideline E6.

The investigator or designated personnel must explain the clinical study and the implications of participation (e.g., objectives, methods, anticipated benefits, and possible risks) to potential subjects according to applicable regulations prior to any clinical study related activity. Subjects will be informed that their participation is voluntary and that they may withdraw from the clinical study at any time. They will be informed that choosing not to participate or to withdraw from the clinical study will not have an impact on the care the subject will receive for the treatment of his/her disease. In case the subject is unable to read and write, an impartial witness must confirm the informed consent.

Background therapy: -

Evidence for comparator: -

| | |
|---|---|
| Actual start date of recruitment | 02 July 2018 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | No |

Notes:

## Population of trial subjects

### Subjects enrolled per country

| | |
|---|---|
| Country: Number of subjects enrolled | United Kingdom: 99999 |
| Worldwide total number of subjects | 99999 |
| EEA total number of subjects | 99999 |

Notes:

### Subjects enrolled per age group

| | |
|---|---|
| In utero | 0 |
| Preterm newborn - gestational age < 37 wk | 0 |
| Newborns (0-27 days) | 0 |

| | |
|---|---|
| Infants and toddlers (28 days-23 months) | 0 |
| Children (2-11 years) | 0 |
| Adolescents (12-17 years) | 0 |
| Adults (18-64 years) | 99999 |
| From 65 to 84 years | 0 |
| 85 years and over | 0 |

## Subject disposition

### Recruitment

Recruitment details:

99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial.

### Pre-assignment

Screening details:

N/A

### Period 1

| | |
|---|---|
| Period 1 title | Overall Trial (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Randomised - controlled |
| Blinding used | Double blind |
| Roles blinded | Investigator, Subject, Monitor, Data analyst, Carer, Assessor |

### Arms

| | |
|---|---|
| **Arm title** | Part I |

Arm description:

F508del homozygous

| | |
|---|---|
| Arm type | Experimental |
| Investigational medicinal product name | GLPG3067 |
| Investigational medicinal product code | G914167 |
| Other name | |
| Pharmaceutical forms | Film-coated tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Not applicable due to trial discontinuation.

| | |
|---|---|
| Investigational medicinal product name | GLPG2222 |
| Investigational medicinal product code | G957389 |
| Other name | |
| Pharmaceutical forms | Tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Not applicable due to trial discontinuation.

| | |
|---|---|
| Investigational medicinal product name | GLPG2737 |
| Investigational medicinal product code | G1117337 |
| Other name | |
| Pharmaceutical forms | Capsule |
| Routes of administration | Oral use |

Dosage and administration details:

Not applicable due to trial discontinuation.

| **Number of subjects in period 1** | Part I |
|---|---|
| Started | 99999 |
| Completed | 99999 |

## Baseline characteristics

### Reporting groups

| Reporting group title | Overall Trial |
|---|---|

Reporting group description: -

| Reporting group values | Overall Trial | Total | |
|---|---|---|---|
| Number of subjects | 99999 | 99999 | |
| Age categorical<br>Units: Subjects | | | |
| In utero | 0 | 0 | |
| Preterm newborn infants (gestational age < 37 wks) | 0 | 0 | |
| Newborns (0-27 days) | 0 | 0 | |
| Infants and toddlers (28 days-23 months) | 0 | 0 | |
| Children (2-11 years) | 0 | 0 | |
| Adolescents (12-17 years) | 0 | 0 | |
| Adults (18-64 years) | 99999 | 99999 | |
| From 65-84 years | 0 | 0 | |
| 85 years and over | 0 | 0 | |
| Age continuous<br>Units: years | | | |
| arithmetic mean | 0 | | |
| standard deviation | ± 0 | - | |
| Gender categorical<br>Units: Subjects | | | |
| Female | 99999 | 99999 | |
| Male | 0 | 0 | |

## End points

### End points reporting groups

| | |
|---|---|
| Reporting group title | Part I |
| Reporting group description: | |
| F508del homozygous | |

### Primary: Change from baseline in sweat chloride concentration

| | |
|---|---|
| End point title | Change from baseline in sweat chloride concentration[1] |
| End point description: | |
| 99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial. | |
| End point type | Primary |
| End point timeframe: | |
| N/A | |

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: No subjects were enrolled in the trial hence results are not available .

| End point values | Part I | | | |
|---|---|---|---|---|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 99999[2] | | | |
| Units: Subjects | | | | |
| Number (not applicable) | 0 | | | |

Notes:

[2] - No subjects were enrolled in the trial hence results are not available

### Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information[1]

Timeframe for reporting adverse events:

From first study drug administration until their follow-up visit.

| | |
|---|---|
| Assessment type | Systematic |

### Dictionary used

| | |
|---|---|
| Dictionary name | MedDRA |
| Dictionary version | 0 |

### Reporting groups

| | |
|---|---|
| Reporting group title | Part I |

Reporting group description: -

| **Serious adverse events** | Part I | | |
|---|---|---|---|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 99999 (0.00%) | | |
| number of deaths (all causes) | 0 | | |
| number of deaths resulting from adverse events | 0 | | |

Frequency threshold for reporting non-serious adverse events: 5 %

| **Non-serious adverse events** | Part I | | |
|---|---|---|---|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 0 / 99999 (0.00%) | | |

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.
Justification: 99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial.

## More information

## Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

## Interruptions (globally)

Were there any global interruptions to the trial? No

## Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

99999 is "Not applicable" value or 0 participants, this trial was discontinued with no participants enrolled in the trial.

Notes: