# Clinical trial results:
# Nab-Paclitaxel (Abraxane®) and Gemcitabine as first line therapy in patients with cholangiocarcinoma ineligible for cisplatin-based chemotherapy – a pilot study
# The NACHO trial (GEMNABCCC-001)

## Summary

| | |
|---|---|
| EudraCT number | 2014-004981-52 |
| Trial protocol | DE |
| Global end of trial date | 10 October 2019 |

**Results information**

| | |
|---|---|
| Result version number | v1 (current) |
| This version publication date | 15 August 2022 |
| First version publication date | 15 August 2022 |
| Summary attachment (see zip file) | CSR_2014-004981-52 (NACHO_CSR_2022-07-29.pdf) |

## Trial information

**Trial identification**

| | |
|---|---|
| Sponsor protocol code | GEMNABCCC-001 |

**Additional study identifiers**

| | |
|---|---|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | - |
| WHO universal trial number (UTN) | - |
| Other trial identifiers | AX-CL-OTHER-PI-003916 : Celgene number |

Notes:

**Sponsors**

| | |
|---|---|
| Sponsor organisation name | University Hospital Essen |
| Sponsor organisation address | Hufelandstr. 55, Essen, Germany, 45147 |
| Public contact | PI, University Hospital Essen, +49 02017231791, gabriele.linden@uk-essen.de |
| Scientific contact | PI, University Hospital Essen, +49 02017231791, gabriele.linden@uk-essen.de |

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 | 01 July 2020 |
| Is this the analysis of the primary completion data? | Yes |
| Primary completion date | 10 October 2019 |
| Global end of trial reached? | Yes |
| Global end of trial date | 10 October 2019 |
| Was the trial ended prematurely? | No |

Notes:

## General information about the trial

Main objective of the trial:

Primary objective:
To determine the efficacy of Gemcitabine/nab-Paclitaxel in first-line therapy of patients with cholangiocarcinoma ineligible for Cisplatin-based therapy. Results of the first 10 evaluable patients will be compared to patient outcomes from the cancer registry of the West German Cancer Center (patients treated with Gemcitabine-combinations, patients treated with monotherapies). Primary endpoint will be overall response rate (ORR) (complete remission and partial remission)

Protection of trial subjects:

AE were measured at every visit.
The regulatory basis of the conduct of this study consisted of the Declaration of Helsinki (in its current version), the AMG [German Medicinal Products Act], in particular Sections 40-42 in the current versions, the guidelines of Good Clinical Practice (ICH-GCP: International Conference on Harmonisation –Good Clinical Practice) valid since 17-Jan-1997, and the GCP-regulation of 09-Aug-2004 (last change of 15-Mar-2006).

Background therapy: -

Evidence for comparator: -

| | |
|---|---|
| Actual start date of recruitment | 04 January 2016 |
| 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 | Germany: 10 |
| Worldwide total number of subjects | 10 |
| EEA total number of subjects | 10 |

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) | 7 |
| From 65 to 84 years | 3 |
| 85 years and over | 0 |

## Subject disposition

### Recruitment

Recruitment details:

Recruitment 06.Dec2016 - 05.Jul 2017

### Pre-assignment

Screening details:

all screened patients were registered. in total, 10 patients started therapy. There was no screen failure

### Period 1

| | |
|---|---|
| Period 1 title | overall trial (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Non-randomised - controlled |
| Blinding used | Not blinded |

### Arms

| | |
|---|---|
| **Arm title** | Nab-Paclitaxel and Gemcitabine |

Arm description:

Nab-Paclitaxel (Abraxane®) and Gemcitabine

| | |
|---|---|
| Arm type | Experimental |
| Investigational medicinal product name | nab-paclitaxel |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Infusion |
| Routes of administration | Intravenous use |

Dosage and administration details:

Nab-Paclitaxel (Abraxane®)
125mg/m² i.v. day 1, 8, 15

for patients with bilirubin 1.5-3ULN
Nab-Paclitaxel (Abraxane®)
100mg/m² i.v. day 1, 8, 15

| | |
|---|---|
| Investigational medicinal product name | Gemcitabine |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Infusion |
| Routes of administration | Intravenous use |

Dosage and administration details:

Gemcitabine
1000mg/m² i.v. day 1, 8, 15

repeat the cycle at day 28, until disease progression

For Patients with elevated bilirubin values >1.5-3x ULN):
Gemcitabine
800mg/m² i.v. day 1, 8, 15

| **Number of subjects in period 1** | Nab-Paclitaxel and Gemcitabine |
|---|---|
| Started | 10 |
| Completed | 10 |

## Baseline characteristics

## End points

### End points reporting groups

| | |
|---|---|
| Reporting group title | Nab-Paclitaxel and Gemcitabine |
| Reporting group description: Nab-Paclitaxel (Abraxane®) and Gemcitabine | |
| Subject analysis set title | ITT |
| Subject analysis set type | Intention-to-treat |
| Subject analysis set description: Intention-To-treat population | |

### Primary: Objective Response Rate (ORR)

| | |
|---|---|
| End point title | Objective Response Rate (ORR)[1] |
| End point description: Altogether 10 patients were enrolled into the trial and received at least one cycle of chemotherapy (ITT-population), In total 4 of the ITT set had CR or PR as best response; this amounted to an ORR of 40%. DCR (CR+PR+SD) was 80%. | |
| End point type | Primary |
| End point timeframe: Registration to EOS | |

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: see full data set

| End point values | ITT | | | |
|---|---|---|---|---|
| Subject group type | Subject analysis set | | | |
| Number of subjects analysed | 10 | | | |
| Units: 4 | | | | |
| number (not applicable) | 10 | | | |

### Statistical analyses

No statistical analyses for this end point

## Adverse events

| Adverse events information[1] | |
|---|---|
| Timeframe for reporting adverse events: Screening to EOS | |
| Assessment type | Systematic |

| Dictionary used | |
|---|---|
| Dictionary name | MedDRA |
| Dictionary version | 3.0 |

Frequency threshold for reporting non-serious adverse events: 1 %

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: see full data set

## 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

None reported