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# Study of INT-747 as monotherapy in patients with primary biliary cirrhosis (PBC)

| Submission date | Recruitment status | [ ] Prospectively registered |
|---|---|---|
| 03/07/2008 | No longer recruiting | [ ] Protocol |
| **Registration date** | **Overall study status** | [ ] Statistical analysis plan |
| 13/08/2008 | Completed | [X] Results |
| **Last Edited** | **Condition category** | [ ] Individual participant data |
| 20/10/2026 | Digestive System | |

**Plain English summary of protocol**
Not provided at time of registration

## Contact information

**Type(s)**
Scientific

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## Additional identifiers

**ClinicalTrials.gov (NCT)**
NCT00570765

**Protocol serial number**
747-201

## Study information

## Scientific Title
A study of INT-747 (6-ethyl chenodeoxycholic acid [6-ECDCA]) monotherapy in patients with primary biliary cirrhosis (PBC)

## Study objectives
The primary hypothesis is that INT-747 will cause a reduction in alkaline phosphatase levels in primary biliary cirrhosis (PBC) patients, over a 12 week treatment period, as compared to placebo.

## Ethics approval required
Old ethics approval format

## Ethics approval(s)
Ethics approval received from:
1. USA: Virginia Commonwealth University, McGuire Institutional Review Board, McGuire VA Medical Centre, 02/10/2007, ref: 01379
2. UK: Multicentre Research Ethics Committee (MREC)
3. Austria: Ethikkommission der Medizinischen Universität Graz, 27/10/2008, ref: 20-003 ex 08/09
4. France: CPP Ile de France VI, 09/07/2008, ref: 47-08
5. Germany: Ethik-Kommission der Medizinischen Hochschule Hannover, 22/04/2009, ref: 5164M
6. Spain: Comitè Ètic Investigació Clínica, 30/06/2008, ref: 747-201

Ethics approval pending from:
7. The Netherlands

All other centres within recruiting countries will seek ethics approval before recruiting participants.

## Primary study design
Interventional

## Study design
Treatment randomised double-blind (subject, investigator) placebo-controlled parallel-assignment safety/efficacy study

## Study type(s)
Treatment

## Health condition(s) or problem(s) studied
Primary biliary cirrhosis

## Interventions
1. Experimental treatment: INT-747 10 mg orally (po) once daily (QD)
2. Experimental treatment: INT 747 50 mg po QD
3. Matched placebo comparator: placebo po QD

Screening period can be up to 4 weeks. Treatment is 12 weeks. Follow up after treatment is 2 weeks.

## Intervention Type
Drug

### Phase
Not Applicable

### Drug/device/biological/vaccine name(s)
INT-747 (6-ethyl chenodeoxycholic acid [6-ECDCA])

### Primary outcome(s)
To assess the effects of INT-747 on:
1. Alkaline phosphatase (AP) levels
2. Safety

Time frame: 12 weeks

### Key secondary outcome(s)
1. To assess the effects of INT-747 on:
1.1. Hepatocellular injury and liver function
1.2. Disease-specific and general health symptoms
1.3. Biomarkers of hepatic inflammation and fibrosis
2. Plasma trough concentrations of INT-747 and its major, known metabolites

Time frame: 12 weeks

### Completion date
31/12/2014

## Eligibility

### Key inclusion criteria
1. Male or female age 18 to 70 years
2. Female patients must be postmenopausal, surgically sterile, or prepared to use two methods of contraception with all sexual partners during the study and for 14 days after the end of dosing
3. Male patients must be prepared to use two methods of contraception with all sexual partners during the study and for 14 days after the end of the dosing
4. Proven or likely PBC, as demonstrated by the patient presenting with at least two of the following three diagnostic factors:
4.1. History of increased AP levels for at least 6 months prior to Day 0
4.2. Positive antimitochondrial antibody (AMA) titre (greater than 1:40 titre on immunofluorescence or M2 positive by enzyme-linked immunosorbent assay [ELISA]) or PBC-specific antinuclear antibodies (antinuclear dot and nuclear rim positive)
4.3. Liver biopsy consistent with PBC
5. Screening alkaline phosphatase (AP) value between 1.5 and 10 x upper limit of normal (ULN)

### Participant type(s)
Patient

### Healthy volunteers allowed
No

### Age group
Mixed

### Lower age limit
18 Years

### Upper age limit
70 Years

### Sex
All

### Total final enrolment
59

### Key exclusion criteria
1. Administration of the following drugs at any time during the three months prior to screening for the study:
1.1. Ursodeoxycholic acid (UDCA, URSO®)
1.2. Colchicine
1.3. Methotrexate
1.4. Azathioprine
1.5. Systemic corticosteroids
2. Screening conjugated (direct) bilirubin greater than 2 x ULN
3. Screening alanine aminotrasferase (ALT) or aspartate aminotrasnferase (AST) greater than 5 x ULN
4. Screening serum creatinine greater than 133 µmol/L (1.5 mg/dL)
5. History or presence of hepatic decompensation (e.g., variceal bleeds, encephalopathy, or poorly controlled ascites)
6. History or presence of other concomitant liver diseases including hepatitis due to hepatitis B or C virus (HBV, HCV) infection, primary sclerosing cholangitis (PSC), alcoholic liver disease, definite autoimmune liver disease or biopsy proven nonalcoholic steatohepatitis (NASH)
7. Pregnancy

### Date of first enrolment
01/11/2007

### Date of final enrolment
31/12/2014

## Locations

### Countries of recruitment
Austria

Canada

France

Germany

Netherlands

Spain

United States of America

**Study participating centre**
**Intercept Pharmaceuticals**
San Diego
United States of America
92122

# Sponsor information

**Organisation**
Intercept Pharmaceuticals (USA)

**ROR**
https://ror.org/01sx6jc36

# Funder(s)

**Funder type**
Industry

**Funder Name**
Genextra S.p.A. (Italy)

**Funder Name**
Visium (USA)

**Funder Name**
JAFCO Life Science Investment (Japan)

# Results and Publications

**Individual participant data (IPD) sharing plan**

## IPD sharing plan summary

Not provided at time of registration

## Study outputs

| Output type | Details | Date created | Date added | Peer reviewed? | Patient-facing? |
|---|---|---|---|---|---|
| Results article | | 29/01/2018 | 20/10/2026 | Yes | No |
| Abstract results | | 01/09/2011 | | No | No |
| Basic results | | | | No | No |