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Study of INT-747 in combination with ursodeoxycholic acid (UDCA [URSO®]) in patients with primary biliary cirrhosis (PBC)

Submission date 03/07/2008	**Recruitment status** No longer recruiting	[_] Prospectively registered [_] Protocol [_] Statistical analysis plan [X] Results [_] Individual participant data
Registration date 13/08/2008	**Overall study status** Completed	
Last Edited 27/04/2018	**Condition category** Digestive System	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number
NCT00550862

Secondary identifying numbers
747-202

Study information

Scientific Title
A study of INT-747 (6-ethyl chenodeoxycholic acid [6-ECDCA]) in combination with ursodeoxycholic acid (UDCA [URSO®]) in patients with primary biliary cirrhosis (PBC)

Study objectives
The primary hypothesis is that INT-747 will cause a reduction in alkaline phosphatase (AP) 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: Institutional Review Board, Beth Israel Medical Centre on the 2nd October 2007 (ref: 133-07)
2. Canada: University of Toronto, University Health Network Research Ethics Board on the 10th June 2008 (ref: 07-0624-A)

Ethics approval received from (as of 09/12/2009):
3. Austria: Ethikkommission der Medizinischen Universität Graz on the 1st October 2008 (ref: 19-316 ex 07/09)
4. France: CPP Ile de France VI on the 27th October 2008 (ref: 85-08)
5. Germany: Ethik-Kommission der Medizinischen Hochschule Hannover on the 17th December 2008 (ref: 5162M)
6. Spain: Comitè Ètic Investigació Clínica on the 19th September 2008 (ref: 747-202)

Ethics approval pending from:
7. UK: Multicentre Research Ethics Committee (MREC)
8. The Netherlands
9. Italy

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

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

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)

Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the sponsor details to request a patient information sheet

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 25 mg po QD
3. Experimental treatment INT-747 50 mg po QD
4. Matched placebo comparator: placebo po QD

Screening can last up to 4 weeks. Treatment is 12 weeks. Follow up after treatment is 2 weeks. Ursodeoxycholic acid (UDCA) treatment is prescribed by each patient's physician; the UDCA dose and timing of its administration each day is determined by each patient's physician (not by the protocol).

Intervention Type
Drug

Phase
Not Applicable

Drug/device/biological/vaccine name(s)
INT-747 (6-ethyl chenodeoxycholic acid [6-ECDCA]), ursodeoxycholic acid (UDCA [URSO®])

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

Time frame: 12 weeks

Secondary outcome measures
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

Overall study start date
01/11/2007

Completion date
02/12/2010

Eligibility

Key inclusion criteria
1. Male or female age 18 to 70 years
2. Stable dose of ursodeoxycholic acid (UDCA [URSO®]) for at least six months prior to screening
3. Female patients must be post-menopausal, 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
4. 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
5. Proven or likely PBC, as demonstrated by the patient presenting with at least two of the following three diagnostic factors:
5.1. History of increased AP levels for at least 6 months prior to Day 0
5.2. 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)
5.3. Liver biopsy consistent with PBC
6. Screening AP value between 1.5 and 10 x upper limit of normal (ULN)

Participant type(s)
Patient

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
140

Key exclusion criteria
1. Administration of the following drugs at any time during the three months prior to screening for the study:
1.1. Colchicine
1.2. Methotrexate
1.3. Azathioprine
1.4. Systemic corticosteroids
2. Screening conjugated (direct) bilirubin greater than 2 x ULN
3. Screening alanine aminotransferase (ALT) or aspartate aminotransferase (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
02/12/2010

Locations

Countries of recruitment
Austria

Canada

France

Germany

Italy

Netherlands

Spain

United Kingdom

United States of America

Study participating centre
Intercept Pharmaceuticals
San Diego
United States of America
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Sponsor information

Organisation
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Sponsor type
Industry

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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

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

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?
Basic results				No	No
Results article	results	01/04/2015		Yes	No