



# Phase 3 study to evaluate the efficacy and safety of human plasma-derived fibrinogen concentrate (FIB Grifols) in subjects with congenital afibrinogenaemia and severe hypofibrinogenemia

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## Main Information

**Primary registry identifying number**

LBCTR2021024523

**Protocol number**

GC1801

**MOH registration number**

**Study registered at the country of origin**

No

**Study registered at the country of origin: Specify**

NA

**Type of registration**

Prospective

**Type of registration: Justify**

N/A

**Date of registration in national regulatory agency**

14/07/2020

**Primary sponsor**

Instituto Grifols, S.A.

**Primary sponsor: Country of origin**

Spain

**Date of registration in primary registry**

21/03/2021

**Date of registration in national regulatory agency**

14/07/2020

**Public title**

Phase 3 study to evaluate the efficacy and safety of human plasma-derived fibrinogen concentrate (FIB Grifols) in subjects with congenital afibrinogenaemia and severe hypofibrinogenemia

**Acronym**

NA

**Scientific title**

A prospective, multicenter, open-label, single-arm study to evaluate the efficacy and safety of human plasma-derived fibrinogen concentrate (FIB Grifols) in subjects with congenital afibrinogenaemia and severe hypofibrinogenemia requiring either ondemand treatment for acute bleeding or surgical prophylaxis

**Acronym**

NA

**Brief summary of the study: English**

This is a phase 3, multi-center, prospective, open-label, single-arm, clinical trial to be carried out in subjects with congenital fibrinogen deficiency manifested as afibrinogenemia or severe hypofibrinogenemia in order to evaluate the efficacy and safety of human plasma-derived fibrinogen concentrate (FIB Grifols)

**Brief summary of the study: Arabic**

هذه دراسة سريرية مستقبلية في المرحلة الثالثة، متعددة المراكز، مفتوحة التسمية، أحادية المجموعة يتم إجراؤها لدى الأشخاص الذين يعانون من نقص الفيبرينوجين الخلقي الذي يتجلى على أنه فيبرينوجين الدم المنعدم أو نقص فيبرينوجين الدم الوخيم من أجل تقييم فعالية وسلامة مركز الفيبرينوجين المشتق من البلازما البشرية (FIB Grifols)

**Health conditions/problem studied: Specify**

Congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia)





## Interventions: Specify

Experimental: FIB Grifols

FIB Grifols will be administered via slow intravenous (IV) infusion at a rate not to exceed 5 mL/minute. Dosing will be individually calculated for each subject based on the target plasma fibrinogen level according to the type of bleeding, measured actual plasma fibrinogen level before infusion, and body weight. The IP will be administered according to the nominal potency of the product.

The duration of FIB Grifols treatment is 1 day with additional IV infusions administered according to daily assessments, clinical trial protocol guidance, and investigator's judgment.

## Key inclusion and exclusion criteria: Inclusion criteria

A subject must meet all the following inclusion criteria to be eligible for participation in this study.

1. Male or female subject between 6 to 70 years of age.
2. Signed and dated written ICF, or the subject's parent or legal guardian signs and dates the ICF where applicable, and the Subject Authorization Form (SAF) where applicable. Pediatric subjects, as defined by local regulations, will be asked to sign an age appropriate assent form.
3. Diagnosed with congenital fibrinogen deficiency manifested as afibrinogenemia or severe hypofibrinogenemia (fibrinogen <50 mg/dL) and expected to require treatment for acute bleeding (either spontaneous or after trauma [defined as any accidental event leading to acute bleeding]), or prophylaxis of bleeding before a surgical intervention or invasive procedure.
4. Fibrinogen level < 50 mg/dL determined by Clauss method at baseline (sample drawn within 24 hours prior to infusion on Infusion 1 Day 1 Visit).
5. Female subjects of child-bearing potential must have a negative test for pregnancy blood or urine human chorionic gonadotropin (HCG)-based assay at baseline (sample drawn within 24 hours prior to infusion on Infusion 1 Day 1 Visit).
  - a. Female subjects/partners of child-bearing potential include any female who has experienced menarche and who has not undergone successful surgical sterilization (eg, hysterectomy, bilateral tubal ligation, or bilateral oophorectomy) or are not postmenopausal (postmenopausal is defined as amenorrhea for >12 consecutive months or women on hormone replacement therapy with documented serum follicle stimulating hormone level <35 mIU/mL). Even women who are using oral, implanted or injectable contraceptive hormones, mechanical products such as an intrauterine device [IUD], or barrier methods (eg, diaphragm, condoms, spermicides) to prevent pregnancy, or are practicing abstinence or where their partner is sterile (eg, vasectomy), should be considered to be of child bearing potential.
6. Willing to comply with all aspects of the clinical trial protocol, including blood sampling, for the entire duration of the study.

## Key inclusion and exclusion criteria: Gender

Both

## Key inclusion and exclusion criteria: Specify gender

## Key inclusion and exclusion criteria: Age minimum

6

## Key inclusion and exclusion criteria: Age maximum

70

## Key inclusion and exclusion criteria: Exclusion criteria

### Exclusion Criteria

A subject meeting any of the following exclusion criteria is NOT eligible for participation in the study.

1. Has acquired (secondary) fibrinogen deficiency.
2. Diagnosed with dysfibrinogenemia.
3. Has known antibodies against fibrinogen.
4. Has history of anaphylaxis or severe systemic response to any drug or blood-derived product.
5. Has history of intolerance to any component of the IP.
6. Documented history of immunoglobulin A (IgA) deficiency and antibodies against IgA.
7. Is a female who is pregnant, breastfeeding or, if of child-bearing potential, unwilling to practice a highly effective method of contraception (eg, oral, injectable, or implantable hormonal methods of contraception, placement of an IUD or intrauterine system, condom or occlusive cap with spermicidal foam/gel/film/cream/suppository, male sterilization, or true abstinence) throughout the study.
  - a. True abstinence: when this is in line with the preferred and usual lifestyle of the subject (Periodic abstinence [eg, calendar, ovulation, symptothermal, post-ovulation methods], declaration of abstinence for the duration of a trial, and withdrawal are not acceptable methods of contraception.
8. Has any medical condition which is likely to interfere with the evaluation of the IP and/or the satisfactory conduct of the clinical trial according to the investigator's judgment.
9. Has congenital or acquired bleeding disorders other than congenital fibrinogen deficiency.
10. Has life expectancy of less than 6 months.
11. Received FRT within 21 days prior to the Screening Visit.
12. Receiving, or having received within 3 months prior to the Screening Visit of this clinical trial, any investigational drug or device.
13. Is unlikely to adhere the protocol requirements, or is likely to be uncooperative, or unable to provide a storage sample prior to IP infusion.

## Type of study

Interventional

## Type of intervention

Pharmaceutical

## Type of intervention: Specify type

N/A

## Trial scope

## Trial scope: Specify scope



Therapy

N/A

**Study design: Allocation**

Single Arm Study

**Study design: Masking**

Open (masking not used)

**Study design: Control**

N/A

**Study phase**

3

**Study design: Purpose**

Treatment

**Study design: Specify purpose**

N/A

**Study design: Assignment**

Single

**Study design: Specify assignment**

N/A

**IMP has market authorization**

No

**IMP has market authorization: Specify**

**Name of IMP**

FIB Grifols

**Year of authorization**

**Month of authorization**

**Type of IMP**

Plasma derived

**Pharmaceutical class**

Human plasma-derived fibrinogen concentrate

**Therapeutic indication**

Congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia)

**Therapeutic benefit**

The intended benefit of fibrinogen concentrates is to avoid hemorrhagic manifestations in subjects with congenital fibrinogen deficiency.

**Study model**

N/A

**Study model: Explain model**

N/A

**Study model: Specify model**

N/A

**Time perspective**

N/A

**Time perspective: Explain time perspective**

N/A

**Time perspective: Specify perspective**

N/A

**Target follow-up duration**

**Target follow-up duration: Unit**

**Number of groups/cohorts**

**Biospecimen retention**

None retained

**Biospecimen description**



NA

**Target sample size**

32

**Actual enrollment target size**

**Date of first enrollment: Type**

Anticipated

**Date of first enrollment: Date**

12/10/2020

**Date of study closure: Type**

Anticipated

**Date of study closure: Date**

31/12/2023

**Recruitment status**

Pending

**Recruitment status: Specify**

**Date of completion**

**IPD sharing statement plan**

No

**IPD sharing statement description**

NA

**Additional data URL**

**Admin comments**

**Trial status**

Approved

## Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Food and Drug Administration	IND number 15811

## Sources of Monetary or Material Support

Name
Instituto Grifols, S.A.



## Secondary Sponsors

Name

NA

## Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Aziz Zoghbi	MCT-CRO	Lebanon	+961 71 008 269	zog_az@mct-cro.com	Country Oversight and Management Africa, Levant and GCC
Scientific	Claudia Khayat	Hotel Dieu de France Hospital	Lebanon	+961 3 704 864	claudiakhayat@yahoo.fr	Principle Investigator

## Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu de France Hospital	Dr. Claudia Khayat	Pediatric Hematology/Oncology	Approved

## Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	30/06/2020	Nancy Alam	nancy.alam@usj.edu.lb	+961 1 421 000 ext 2335

## Countries of Recruitment

Name

India

Bulgaria

Lebanon

Turkey

United States of America

United Kingdom



## Health Conditions or Problems Studied

Condition	Code	Keyword
Congenital fibrinogen deficiency manifested as afibrinogenemia or severe hypofibrinogenemia	Fibrinolysis-affecting drugs (T45.6)	afibrinogenemia or severe hypofibrinogenemia

## Interventions

Intervention	Description	Keyword
Experimental: FIB Grifols	FIB Grifols will be administered via slow intravenous (IV) infusion at a rate not to exceed 5 mL/minute. Dosing will be individually calculated for each subject based on the target plasma fibrinogen level according to the type of bleeding, measured actual plasma fibrinogen level before infusion, and body weight. The IP will be administered according to the nominal potency of the product. The duration of FIB Grifols treatment is 1 day with additional IV infusions administered according to daily assessments, clinical trial protocol guidance, and investigator's judgment.	FIB Grifols, IV

## Primary Outcomes

Name	Time Points	Measure
overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	The primary efficacy endpoint is the overall clinical assessment of the hemostatic efficacy of FIB Grifols in treating all documented acute bleeding episodes or in preventing excessive bleeding during and after all documented surgical procedures according to a 4-point scale. Hemostatic efficacy will be rated as excellent, good, moderate, or none. The primary efficacy endpoint will be assessed by the IEAC.



## Key Secondary Outcomes

Name	Time Points	Measure
Overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	Overall clinical assessment of the hemostatic efficacy of FIB Grifols in treating the first documented acute bleeding episode or in preventing excessive bleeding during and after the first documented surgical procedure according to a 4 point scale. This secondary efficacy endpoint will be assessed by the IEAC.
Overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	Overall clinical assessment of the hemostatic efficacy of FIB Grifols in treating the first acute bleeding episode as assessed by the principal investigator at each trial site according to a 4-point scale
Overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	Overall clinical assessment of the hemostatic efficacy of FIB Grifols in treating all acute bleeding episodes as assessed by the principal investigator at each trial site according to a 4-point scale
Overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	Clinical assessment of the hemostatic efficacy of FIB Grifols in preventing excessive bleeding intra-operatively for all surgical procedures as assessed by the surgeon (defined as the licensed medical professional performing the invasive procedure [eg, dentist]) according to a 4-point scale
Overall clinical assessment of the hemostatic efficacy of FIB Grifols	In case of bleeding episode: At the end of treatment Observation period visit. In case of surgery: End of surgery and at the end of treatment observational period visit	Clinical assessment of the hemostatic efficacy of FIB Grifols in preventing excessive bleeding after all surgical procedures (ie, post-operative) as assessed by the principal investigator at each trial site according to a 4-point scale
Difference in MCF	From pre-infusion to 1 hour after the end of each infusion	Measured by ROTEM
Difference in MCF	From pre-infusion to 4 hours after the end of each infusion	Measured by ROTEM
Difference (improvement) in Thrombin Time	From pre-infusion to 1 hour after the end of each infusion	Difference (improvement) in Thrombin Time
Difference (improvement) in Prothrombin Time	From pre-infusion to 1 hour after the end of each infusion	Difference (improvement) in Prothrombin Time
Difference (improvement) in activated Partial Thromboplastin Time	From pre-infusion to 1 hour after the end of each infusion	Difference (improvement) in activated Partial Thromboplastin Time
Difference in plasma fibrinogen levels	From pre-infusion to 1 hour after the end of each infusion	By Clauss and ELISA
Difference in plasma fibrinogen levels	From pre-infusion to 4 hours after the end of each infusion	By Clauss and ELISA
Difference in plasma fibrinogen levels	From pre-infusion to 24 hours after the end of each infusion	By Clauss and ELISA
Incremental IVR	During the first 4 hours of sampling after the end of each infusion of FIB Grifols calculated using concentration of plasma fibrinogen	Measured by both Clauss method (functional) and ELISA method (immunologic) for subjects with acute bleeding episodes or need for additional post-operative FIB Grifols infusion



## Trial Results

**Summary results**

**Study results globally**

**Date of posting of results summaries**

**Date of first journal publication of results**

**Results URL link**

**Baseline characteristics**

**Participant flow**

**Adverse events**

**Outcome measures**

**URL to protocol files**