NOVARTIS

Clinical Trial Results Website

Sponsor

Novartis Pharmaceuticals

Generic Drug Name

CSJ137

Trial Indication(s)

Anemia

Protocol Number

CCSJ137X2201

Protocol Title

A first-in-human, two-part (open label, and randomized/double blind/placebo controlled), single- and repeat-dose study of CSJ137 in erythropoietin-treated chronic hemodialysis patients with functional iron-deficiency anemia

Clinical Trial Phase

Phase 1

Phase of Drug Development

I/II

Study Start/End Dates

Study Start Date: September 2015 (Actual)
Primary Completion Date: May 2020 (Actual)
Study Completion Date: May 2020 (Actual)

Reason for Termination (If applicable)

The study was terminated early due to strategic reasons following an internal decision at the end of Part 1 and new patients were not enrolled post the study termination date.

Study Design/Methodology

This was a first-in-human, two-part (open-label, and randomized/double-blind/placebo-controlled), single- and repeat-dose study of CSJ137 in erythropoietin-treated chronic hemodialysis patients with functional iron-deficiency anemia.

Part 1 used an open-label dose-finding design in which the patients received a single dose infusion of CSJ137 on Day 1. A total of 40 patients were assigned to one of the 8 open-label dose cohorts.

In Cohort 1: 0.01mg/kg, participants received a single dose of CSJ137 having lower ferritin inclusion criteria than participants in subsequent cohorts

In Cohort 1: 1.62mg/kg, one participant had a dosing error and was thus reported in a separate group. The participant had lower ferritin inclusion criteria than participants in subsequent cohorts

In Cohort 2: 0.01mg/kg, participants received the same dose level as participants in Cohort 1: 0.01mg/kg group, but had higher ferritin inclusion criteria than participants from Cohort 1

From Cohort 3 to Cohort 8, participants received a single dose of CSJ137 respectively.

The study included:

- a **Screening epoch** of up to 60 days to assess eligibility as per the inclusion and exclusion criteria,
- a **Baseline epoch** of up to 5 days to reevaluate the eligibility criteria before dosing,
- a **CSJ137 dosing** on Day 1 where the patients assessed as eligible during screening and baseline visits were dosed with a single dose infusion of CSJ137 following that day's dialysis session,
- a **Dosing epoch** of up to Day 85, which was the end of the study. The patients were contacted on Day 115 post the study for safety follow-up.

Centers

10 centers in 2 countries: United States(4), Israel(6)

Objectives: Part-1

Primary objectives

- To assess safety and tolerability following a single dose of CSJ137
- To determine the minimum pharmacologically active dose (PAD) of CSJ137

Secondary objective

- To assess pharmacokinetics (PK)

Test Product (s), Dose(s), and Mode(s) of Administration

CSJ137 was provided as liquid solution in vials. No placebo was supplied.

Statistical Methods

The primary variables were adverse events and Hemoglobin (Hgb) response. The Hgb response was determined by levels of hemoglobin in blood, without the subject showing evidence of liver dysfunction or other safety concerns. The number and percentage of patients with Hgb responses were tabulated by cohort/treatment in Part 1. The secondary pharmacokinetic endpoints included evaluation of PK parameters from serum concentration-time data (Cmax and AUClast).

Study Population: Key Inclusion/Exclusion Criteria

Inclusion Criteria:

1. Hemodialysis-dependent for at least 2 months prior to screening.
2. Receiving hemodialysis at least 2 times per week
3. Receiving erythropoietin (EPO) therapy.
4. Hemoglobin (Hgb) ≥ 8.5 and < 11.5 g/dL at screening.
5. Ferritin >500 ng/mL and ≤ 2000 ng/mL at screening.
6. TSAT ≤ 50% at a minimum of one time point during the 90 days prior to baseline.

Exclusion Criteria:
1. Known diagnosis of hemochromatosis, bone marrow malignancy, lymphatic malignancy or myelodysplastic syndrome.
2. History of dialysis AV fistula thrombosis within 2 months prior to screening, or 2 or more episodes of AV fistula thrombosis within 6 months prior to screening.
3. Liver disease/dysfunction (Child-Pugh score ≥ 6), prior liver transplant, heart failure (NYHA Class III or IV); gastrointestinal bleeding.
4. A positive Hepatitis B surface antigen test result. Patients with Hepatitis C Virus (HCV) infection may be included if all other liver function eligibility criteria are met.
5. ALT, AST or bilirubin ≥ 1.5x ULN within 4 weeks prior to baseline.
6. Uncontrolled renal osteodystrophy
7. Conditions predisposing to an increased risk of serious infection, such as an indwelling vascular catheter (central venous line or non-tunneled/acute hemodialysis catheter) or active infection requiring antibiotic therapy at any time during the 2 weeks prior to screening. Tunneled hemodialysis catheters, and other "permanent" catheters are permitted.
8. Blood transfusion administered within 4 weeks prior to baseline.
9. Patients who received CSJ137 dose in the past.

Other protocol-defined inclusion/exclusion criteria may apply.

Participant Flow Table

Overall Study

	Cohort 1: 0.01mg/kg	Cohort 1: 1.62mg/kg	Cohort 2: 0.01mg/kg	Cohort 3: 0.04mg/kg	Cohort 4: 0.10mg/kg	Cohort 5: 0.30mg/kg	Cohort 6: 1.00mg/kg	Cohort 7: 3.00mg/kg	Cohort 8: 10.00mg/kg	Total
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	
Started	5	1	5	3	6	3	6	5	6	40
Pharmacodynamics (PD) analysis set	5	1	5	3	6	3	6	5	6	40
Pharmacokinetics analysis set (PK) analysis set	5	1	5	3	5	3	6	5	6	39

Completed	4	1	5	3	6	3	6	5	6	39
Not Completed	1	0	0	0	0	0	0	0	0	1
Subject/guardian decision	1	0	0	0	0	0	0	0	0	1

Baseline Characteristics

	Cohort 1: 0.01mg/ kg	**Cohort 1: 1.62mg/ kg**	**Cohort 2: 0.01mg/ kg**	**Cohort 3: 0.04mg/ kg**	**Cohort 4: 0.10mg/ kg**	**Cohort 5: 0.30mg/ kg**	**Cohort 6: 1.00mg/ kg**	**Cohort 7: 3.00mg/ kg**	**Cohort 8: 10.00mg/ kg**	**Total**
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	
Number of Participants [units: participants]	5	1	5	3	6	3	6	5	6	40
Age Continuous (units: Years) Mean ± Standard Deviation										
	58.20±8.319	61.00±NA[]	48.20±22.231	56.67±9.452	62.33±11.742	60.00±6.557	59.83±13.212	52.20±10.663	67.17±16.654	58.50±13.610
Sex: Female, Male (units: Participants) Count of Participants (Not Applicable)										
Female	4	0	2	0	2	1	5	3	1	18
Male	1	1	3	3	4	2	1	2	5	22
Race/Ethnicity, Customized (units: Participants) Count of Participants (Not Applicable)										

Other	5	1	5	3	6	3	5	5	6	39
Hispanic/Latino	0	0	0	0	0	0	1	0	0	1

Primary Outcome Result(s)

Number of participants with adverse events and serious adverse events

(Time Frame: Day 1 to Day 85 (end of study))

	Cohort 1: 0.01mg/ kg	Cohort 1: 1.62mg/ kg	Cohort 2: 0.01mg/ kg	Cohort 3: 0.04mg/ kg	Cohort 4: 0.10mg/ kg	Cohort 5: 0.30mg/ kg	Cohort 6: 1.00mg/ kg	Cohort 7: 3.00mg/ kg	Cohort 8: 10.00mg/ kg
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137
Number of Participants Analyzed [units: participants]	5	1	5	3	6	3	6	5	6
Number of participants with adverse events and serious adverse events (units: Participants) Count of Participants (Not Applicable)									
Patients with AEs	5 (100%)	1 (100%)	4 (80%)	3 (100%)	5 (83.33%)	2 (66.67%)	3 (50%)	2 (40%)	3 (50%)
Serious AEs	3 (60%)	0 (%)	2 (40%)	3 (100%)	0 (%)	0 (%)	1 (16.67%)	0 (%)	0 (%)

Number of participants with Hemoglobin (Hgb) response

(Time Frame: Day 29)

	Cohort 1: 0.01mg/ kg	Cohort 1: 1.62mg/ kg	Cohort 2: 0.01mg/ kg	Cohort 3: 0.04mg/ kg	Cohort 4: 0.10mg/ kg	Cohort 5: 0.30mg/ kg	Cohort 6: 1.00mg/ kg	Cohort 7: 3.00mg/ kg	Cohort 8: 10.00mg/ kg
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137

Number of Participants Analyzed [units: participants]	4	1	5	3	6	3	6	4	6
Number of participants with Hemoglobin (Hgb) response (units: Participants) Count of Participants (Not Applicable)									
Hgb response	0 (%)	0 (%)	2 (40%)	1 (33.33%)	2 (33.33%)	1 (33.33%)	1 (16.67%)	0 (%)	1 (16.67%)

Secondary Outcome Result(s)

Peak concentration (Cmax) of CSJ137 in serum

(Time Frame: pre-dose, 0.5 hours and 6 hours post-dose on Day 1, 2, 3, 5, 12, 19, 28, and 84)

	Cohort 1: 0.01mg/ kg	**Cohort 1: 1.62mg/ kg**	**Cohort 2: 0.01mg/ kg**	**Cohort 3: 0.04mg/ kg**	**Cohort 4: 0.10mg/ kg**	**Cohort 5: 0.30mg/ kg**	**Cohort 6: 1.00mg/ kg**	**Cohort 7: 3.00mg/ kg**	**Cohort 8: 10.00mg/ kg**
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137
Number of Participants Analyzed [units: participants]	5	1	5	3	5	3	6	5	6
Peak concentration (Cmax) of CSJ137 in serum (units: ug/mL) Mean ± Standard Deviation									

	0.232 ± 0.193	40.7 ± NA[1]	0.235 ± 0.0585	1.49 ± 1.20	1.88 ± 2.09	4.38 ± 4.02	17.1 ± 3.06	65.7 ± 15.7	197 ± 37.7

[1] Standard deviation not calculated with 1 participant

Measurement of CSJ137 serum concentration and calculation of AUClast

(Time Frame: pre-dose, 0.5 hours and 6 hours post-dose on Day 1, 2, 3, 5, 12, 19, 28, and 84)

	Cohort 1: 0.01mg/ kg	**Cohort 1: 1.62mg/ kg**	**Cohort 2: 0.01mg/ kg**	**Cohort 3: 0.04mg/ kg**	**Cohort 4: 0.10mg/ kg**	**Cohort 5: 0.30mg/ kg**	**Cohort 6: 1.00mg/ kg**	**Cohort 7: 3.00mg/ kg**	**Cohort 8: 10.00mg/ kg**
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137
Number of Participants Analyzed [units: participants]	5	1	5	3	5	3	6	5	6
Measurement of CSJ137 serum concentration and calculation of AUClast (units: day*ug/mL) Mean ± Standard Deviation									
	1.11 ± 1.14	449 ± NA[1]	0.893 ± 0.254	9.19 ± 9.87	11.1 ± 15.0	34.7 ± 37.3	269 ± 72.8	799 ± 145	3030 ± 839

[1] Standard deviation not calculated with 1 participant

Safety Results

All-Cause Mortality

	Cohort 1: 0.01mg/ kg N = 5	Cohort 1: 1.62mg/ kg N = 1	Cohort 2: 0.01mg/ kg N = 5	Cohort 3: 0.04mg/ kg N = 3	Cohort 4: 0.10mg/ kg N = 6	Cohort 5: 0.30mg/ kg N = 3	Cohort 6: 1.00mg/ kg N = 6	Cohort 7: 3.00mg/ kg N = 5	Cohort 8: 10.00mg/ kg N = 6	Total N = 40
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	Total
Total participants affected	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)

Serious Adverse Events by System Organ Class

Time Frame	Adverse events were collected from first dose of study treatment until end of study treatment Day 85 plus 30 days post treatment (Day 115).
Additional Description	Any sign or symptom collected from first dose of study treatment until end of study treatment Day 85 plus 30 days post treatment (Day 115).
Source Vocabulary for Table Default	MedDRA (20.1)
Assessment Type for Table Default	Systematic Assessment

	Cohort 1: 0.01mg/ kg N = 5	Cohort 1: 1.62mg/ kg N = 1	Cohort 2: 0.01mg/ kg N = 5	Cohort 3: 0.04mg/ kg N = 3	Cohort 4: 0.10mg/ kg N = 6	Cohort 5: 0.30mg/ kg N = 3	Cohort 6: 1.00mg/ kg N = 6	Cohort 7: 3.00mg/ kg N = 5	Cohort 8: 10.00mg/ kg N = 6	Total N = 40

Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	Total
Total participants affected	3 (60.00%)	0 (0.00%)	2 (40.00%)	3 (100.00%)	0 (0.00%)	0 (0.00%)	2 (33.33%)	0 (0.00%)	0 (0.00%)	10 (25.00%)
Blood and lymphatic system disorders										
Anaemia	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Cardiac disorders										
Cardiac failure acute	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Coronary artery disease	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	2 (5.00%)
Gastrointestinal disorders										
Large intestinal obstruction	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Hepatobiliary disorders										
Bile duct stone	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Cholelithiasis	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Infections and infestations										
Bacteraemia	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pneumonia	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Injury, poisoning and procedural complications										

Arteriovenous fistula site complication	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Metabolism and nutrition disorders										
Fluid overload	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Hyperkalaemia	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)										
Metastases to spine	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Prostate cancer	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Nervous system disorders										
Myoclonus	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Renal and urinary disorders										
Subcapsular renal haematoma	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Respiratory, thoracic and mediastinal disorders										
Dyspnoea exertional	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Vascular disorders										

Hypotension	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Other Adverse Events by System Organ Class

Time Frame	Adverse events were collected from first dose of study treatment until end of study treatment Day 85 plus 30 days post treatment (Day 115).
Additional Description	Any sign or symptom collected from first dose of study treatment until end of study treatment Day 85 plus 30 days post treatment (Day 115).
Source Vocabulary for Table Default	MedDRA (20.1)
Assessment Type for Table Default	Systematic Assessment
Frequent Event Reporting Threshold	5%

	Cohort 1: 0.01mg/ kg N = 5	**Cohort 1: 1.62mg/ kg N = 1**	**Cohort 2: 0.01mg/ kg N = 5**	**Cohort 3: 0.04mg/ kg N = 3**	**Cohort 4: 0.10mg/ kg N = 6**	**Cohort 5: 0.30mg/ kg N = 3**	**Cohort 6: 1.00mg/ kg N = 6**	**Cohort 7: 3.00mg/ kg N = 5**	**Cohort 8: 10.00mg/ kg N = 6**	**Total N = 40**
Arm/Group Description	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	single dose of CSJ137	Total
Total participants affected	4 (80.00%)	1 (100.00%)	4 (80.00%)	3 (100.00%)	5 (83.33%)	2 (66.67%)	3 (50.00%)	2 (40.00%)	3 (50.00%)	27 (67.50%)
Endocrine disorders										
Hyperparathyroidism secondary	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Gastrointestinal disorders										
Abdominal distension	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Abdominal pain lower	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	1 (2.50%)
Abdominal pain upper	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Constipation	1 (20.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	2 (5.00%)
Dental caries	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	1 (2.50%)
Diarrhoea	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	2 (5.00%)
Duodenitis	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Nausea	1 (20.00%)	0 (0.00%)	2 (40.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	2 (33.33%)	0 (0.00%)	0 (0.00%)	6 (15.00%)
Rectal haemorrhage	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	1 (2.50%)
Vomiting	1 (20.00%)	0 (0.00%)	1 (20.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	3 (7.50%)
General disorders and administration site conditions										
Asthenia	1 (20.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	1 (33.33%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	4 (10.00%)
Chills	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Fatigue	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Gait disturbance	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Non-cardiac chest pain	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Pain	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pyrexia	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Hepatobiliary disorders										
Cholelithiasis	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Infections and infestations										
Fungal skin infection	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Influenza	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pharyngitis	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pustule	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Upper respiratory tract infection	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	2 (33.33%)	0 (0.00%)	0 (0.00%)	3 (7.50%)
Vaginal infection	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Injury, poisoning and procedural complications										
Arteriovenous fistula site complication	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Arteriovenous fistula site haemorrhage	0 (0.00%)	1 (100.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Arteriovenous graft site stenosis	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	2 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	2 (5.00%)
Fall	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	2 (5.00%)
Humerus fracture	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Joint injury	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	1 (2.50%)
Scapula fracture	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	1 (2.50%)
Thermal burn	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Metabolism and nutrition disorders										
Calciphylaxis	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Decreased appetite	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Folate deficiency	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Hyperkalaemia	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Hypervolaemia	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Iron deficiency	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Vitamin B12 deficiency	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Musculoskeletal and connective tissue disorders										
Arthralgia	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	2 (5.00%)

Joint swelling	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	1 (2.50%)
Muscle twitching	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pain in extremity	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Polyarthritis	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	1 (2.50%)
Synovial cyst	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	1 (2.50%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)										
Benign neoplasm of skin	0 (0.00%)	1 (100.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Nervous system disorders										
Headache	0 (0.00%)	0 (0.00%)	1 (20.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Psychiatric disorders										
Anxiety	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Respiratory, thoracic and mediastinal disorders										
Cough	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	1 (16.67%)	2 (5.00%)
Sinus congestion	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Skin and subcutaneous tissue disorders										
Hyperhidrosis	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)
Pruritus	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	1 (16.67%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	2 (5.00%)
Skin ulcer	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (33.33%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	0 (0.00%)	1 (2.50%)

Other Relevant Findings

None

Conclusion:

CSJ137 was well tolerated after a single dose in patients receiving chronic hemodialysis complicated by functional iron deficiency anemia. A single dose of CSJ137 did not increase hemoglobin (Hgb) in the trial participants. Further studies are required to assess safety and efficacy of CSJ137 after multiple doses.

Date of Clinical Trial Report

December 16, 2020