ClinicalTrials.gov Protocol Registration and Results System (PRS) Receipt
Release Date: 06/24/2016

ClinicalTrials.gov ID: NCT00551291

Study Identification

Unique Protocol ID:	ML20559
Brief Title:	A Study of NeoRecormon (Epoetin Beta), CellCept (Mycophenolate Mofetil) and Prednisone in Patients With Low or Intermediate Myelodysplastic Syndromes.
Official Title:	An Open Label Study of the Effects of a Combination of NeoRecormon, CellCept and Prednisone on Hematological Parameters and Cytogenesis in Patients With Low or Intermediate Risk Myelodysplastic Syndromes.¿
Secondary IDs:	

Study Status

Record Verification:	June 2016
Overall Status:	Completed
Study Start:	August 2007
Primary Completion:	June 2009 [Actual]
Study Completion:	June 2009 [Actual]

Sponsor/Collaborators

Sponsor:	Hoffmann-La Roche
Responsible Party:	Sponsor
Collaborators:	

Oversight

FDA Regulated?:	Yes

Applicable Trial?:	Section 801 Clinical Trial? Yes Delayed Posting? No
IND/IDE Protocol?:	No
Review Board:	Approval Status: Approved Approval Number: Unknown Board Name: Comite Etico de Investigacion Clinica del Hospital Clinico San Carlos de Madrid Board Affiliation: CEIC del Hospital Clinico San Carlos de Madrid Phone: +34 913 303 819 Email: ceic.hcsc@salud.madrid.org
Data Monitoring?:	
Plan to Share Data?:	
Oversight Authorities:	Spain: Agencia Española de Medicamentos y Productos Sanitarios

Study Description

Brief Summary:	This single arm study will evaluate the efficacy and safety of a combination of NeoRecormon, CellCept and prednisone in patients with low or moderate risk myelodysplastic syndromes (MDS). In the first phase of the study, patients will receive CellCept (1g p.o. twice daily) plus prednisone. After 3 months, if patients have not responded to treatment, NeoRecormon (30000 IU/week, s.c.) will be added to the treatment regimen. If there is no response to NeoRecormon after 6 weeks, the dose will be increased to 60000 IU/week. The anticipated time on study treatment is 3-12 months, and the target sample size is <100 individuals.
Detailed Description:	

Conditions

Conditions:	Myelodysplastic Syndromes
Keywords:	

Study Design

Study Type:	Interventional
Primary Purpose:	Treatment
Study Phase:	Phase 2
Intervention Model:	Single Group Assignment
Number of Arms:	1

Masking:	Open Label
Allocation:	N/A
Endpoint Classification:	Safety/Efficacy Study
Enrollment:	10 [Actual]

Arms and Interventions

Arms	Assigned Interventions
Experimental: Mycophenolate Mofetil + Prednisone + Erythropoietin Beta Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12.	Drug: Mycophenolate mofetil 1 gm twice daily orally until end of study. Other Names: • CellCept • MMF Drug: Prednisone 10 mg/day orally until end of study. Drug: Erythropoietin Beta Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks. Other Names: • NeoRecormon

Outcome Measures

[See Results Section.]

Eligibility

Minimum Age:	18 Years
Maximum Age:	
Gender:	Both
Accepts Healthy Volunteers?:	No
Criteria:	Inclusion Criteria:

- adult patients, >=18 years of age;
- diagnosis of MDS, according to International Prognostic Scoring System (IPSS) criteria;
- low or intermediate risk, who are not candidates for treatment with growth factors, or who have not responded to these treatments.

Exclusion Criteria:

- previous treatment with CellCept, or any erythropoietin-stimulating drug;
- diagnosis of proliferative chronic myelomonocytic leukemia;
- prior or concomitant malignancies other than MDS, with the exception of basocellular, spinocellular or adequately treated in situ cervical cancer, in the past 3 years;
- biological antitumor and myelosuppressive treatment within 28 days before start of study;
- bone marrow precursor cell transplantation previous to study.

Contacts/Locations

Study Officials: Clinical Trials
Study Director
Hoffmann-La Roche

Locations: Spain

Barcelona, Spain, 08036

Cádiz, Spain, 11009

Madrid, Spain, 28040

Barcelona, Spain, 08035

Barakaldo, Spain, 48903

Barcelona, Spain, 08003

Palma de Mallorca, Spain, 07198

Barcelona, Spain, 08025

References

Citations:

Links:

Study Data/Documents:

Participant Flow

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Overall Study

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Started	10
Completed	9
Not Completed	1
Physician Decision	1

Baseline Characteristics

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Baseline Measures

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Number of Participants	10
Age, Continuous [units: years] Mean (Standard Deviation)	75.00 (5.17)
Gender, Male/Female [units: participants]	
Female	3
Male	7

Outcome Measures

1. Primary Outcome Measure:

Measure Title	Percentage of Participants With Clinical Response as Measured by the International Working Group (IWG) Criteria for Hematological Improvement
Measure Description	International Working Group (IWG) criteria for hematological improvement was defined as having hemoglobin (Hgb) <11 g/dL (pretreatment) and an increase in Hgb ≥1.5 g/dL after ≥8 weeks of treatment.
Time Frame	Up to approximately 2 years
Safety Issue?	No

Analysis Population Description

[Not Specified]

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Measured Values

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Number of Participants Analyzed	10
Percentage of Participants With Clinical Response as Measured by the International Working Group (IWG) Criteria for Hematological Improvement [units: percentage of participants]	
Week 12 (n=4)	50.00
Week 18 (n=7)	71.43
End of study (n=3)	100.00

2. Primary Outcome Measure:

Measure Title	Mean Number of Blood Transfusions Per Visit
Measure Description	
Time Frame	Up to approximately 2 years
Safety Issue?	No

Analysis Population Description

[Not Specified]

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Measured Values

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Number of Participants Analyzed	10

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Mean Number of Blood Transfusions Per Visit [units: transfusions/visit] Mean (Standard Deviation)	
Baseline (n=8)	4.13 (2.30)
Week 12 (n=6)	5.83 (2.86)
Week 18 (n=5)	2.80 (1.92)
End of Study (n=3)	2.33 (1.53)

3. Secondary Outcome Measure:

Measure Title	Percentage of Participants With at Least One Adverse Event (AE)
Measure Description	An AE was considered any unfavorable and unintended sign, symptom, or disease associated with the use of the study drug, whether or not considered related to the study drug. Preexisting conditions that worsened during the study and laboratory or clinical tests that resulted in a change in treatment or discontinuation from study drug were reported as adverse events.
Time Frame	Up to approximately 2 years
Safety Issue?	No

Analysis Population Description

[Not Specified]

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Measured Values

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
Number of Participants Analyzed	10
Percentage of Participants With at Least One Adverse Event (AE) [units: percentage of participants]	90.00

Reported Adverse Events

Time Frame	Up to approximately 2 years.
Additional Description	[Not specified]

Reporting Groups

	Description
Mycophenolate Mofetil + Prednisone + Erythropoietin Beta	Mycophenolate mofetil (MMF) 1 gm twice daily orally and prednisone 10 mg/day orally until the end of the study. Recombinant human erythropoietin beta 30,000 IU/week, subcutaneously for 6 weeks was added in case of no significant response at Week 12. Mycophenolate mofetil: 1 gm twice daily orally until end of study. Prednisone: 10 mg/day orally until end of study. Erythropoietin Beta: Recombinant human erythropoietin beta at doses of 30,000 IU/week by the subcutaneous route for 6 weeks.

Serious Adverse Events

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
	Affected/At Risk (%)
Total	4/10 (40%)
Cardiac disorders	
Cardiac insufficiency [A] †	1/10 (10%)
Gastrointestinal disorders	
Rectal bleeding [A] †	1/10 (10%)

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
	Affected/At Risk (%)
Hepatobiliary disorders	
Colecistitis [A] †	1/10 (10%)
Infections and infestations	
Bronchitis [A] †	1/10 (10%)
Pneumonia [A] †	2/10 (20%)
Musculoskeletal and connective tissue disorders	
Achilles tendon´s break [A] †	1/10 (10%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA 17.0

Other Adverse Events

Frequency Threshold Above Which Other Adverse Events are Reported: 5%

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
	Affected/At Risk (%)
Total	9/10 (90%)
Blood and lymphatic system disorders	
Splenomegaly increase [A] †	1/10 (10%)
Cardiac disorders	
Dyspnea on exertion [A] †	1/10 (10%)
Dyspnea to great efforts [A] †	1/10 (10%)
Palpitations [A] †	1/10 (10%)
Endocrine disorders	
Decompensated diabetes mellitus [A] †	1/10 (10%)
Gastrointestinal disorders	
Diarrhea [A] †	2/10 (20%)

	Mycophenolate Mofetil + Prednisone + Erythropoietin Beta
	Affected/At Risk (%)
Epigastralgia [A] †	1/10 (10%)
Gastroenteritis [A] †	1/10 (10%)
General disorders	
Asthenia [A] †	2/10 (20%)
Dysthermia feeling [A] †	1/10 (10%)
Infections and infestations	
Bronchial infection [A] †	1/10 (10%)
Herpes labialis [A] †	1/10 (10%)
Influenza [A] †	1/10 (10%)
Respiratory infection [A] †	1/10 (10%)
Upper respiratory tract catarrh [A] †	3/10 (30%)
Injury, poisoning and procedural complications	
Chest injury due to fall [A] †	1/10 (10%)
Musculoskeletal and connective tissue disorders	
Achilles tendon´s tendinitis [A] †	1/10 (10%)
Coxalgia [A] †	1/10 (10%)
Psychiatric disorders	
Nervousness [A] †	1/10 (10%)
Respiratory, thoracic and mediastinal disorders	
Occasional tightness in the chest [A] †	1/10 (10%)
Skin and subcutaneous tissue disorders	
Facial allergic reaction [A] †	1/10 (10%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA 17.0

Limitations and Caveats

[Not specified]

More Information

Certain Agreements:

Principal Investigators are NOT employed by the organization sponsoring the study.

There IS an agreement between the Principal Investigator and the Sponsor (or its agents) that restricts the PI's rights to discuss or publish trial results after the trial is completed.

The Study being conducted under this Agreement is part of the Overall Study. Investigator is free to publish in reputable journals or to present at professional conferences the results of the Study, but only after the first publication or presentation that involves the Overall Study. The Sponsor may request that Confidential Information be deleted and/or the publication be postponed in order to protect the Sponsor's intellectual property rights.

Results Point of Contact:

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