CURRICULUM VITAE Lucia De Franceschi

Education

- **1991.** Medical degree at the University of Verona: 110/110 cum laude (presenter prof R Corrocher)
- **1997.** Specialization in Internal Medicine, University of Verona,: 50/50 cum laude (presenters: prof G De Sandre and prof R Corrocher).

Scientific and professional history

- **1991-1992. Research fellow** at the Department of Physiology, harvard Medical School, Boston, MA, USA
- **1993 to 1997**. During her internship in Internal Medicine (Dept. of Medicine, Section of Internal Medicine, University of Verona she spent **3 months per year as visiting researcher** Unité de Recherche en genetique moleculaire et en Hematologie, INSERM U91, Hopital H Mondor, Universitè de Creteil, France, Direttore: prof. Y. Beuzard e presso Laboratory of Gene Therapy, Hopital S Louis, Paris, France, Director: prof. Y. Beuzard.
- **1997-2000.** She was a member of the **European steering Commitee-BIOMED 2-PL95** on project entitled: “Reactivation of fetal hemoglobin synthesis as a means to improve the quality of life of patients with hemoglobinopathies”.
- **01.03.1998-30.08.1998. Visiting Instructor in Pathology** ,Department of Pathology, Children’s Hospital, Harvard Medical School, Boston, MA, USA. Director: prof. C Brugnara.
- **September 1998 she became Researcher in Internal Medicine (MED09)** with a permanent position in the Dept. of Clinical and Experimental Medicine, Section of Internal Medicine, University of Verona.
- **From 2003** until now she is in the panel review of the **Cochrane cystic fibrosis and genetic disorders group** on sickle cell disease and related clinical and therapeutic issues.
- **From 2002** until now she is ia member of the teaching staff of the PhD program on Clinical Proteomics and she is in charged for the educational and tutorial programs.

- In **February 2006** she obtained the qualification of associated professor in Internal Medicine (MED/09) and in **October 2006** she got the permanent position Dept. of Clinical and Experimental Medicine, Section of Internal Medicine, University of Verona.
- From Nov. 2000 to Nov. 2011 and from Feb 2015 up-to now, she had an **annual appointment as Associated Researcher** Depts of Pathology and Laboratory of Medicine, Children's Hospital, Harvard Medical School, Boston, MA, USA.
- From 2006-2023 she was Associated professor of Internal Medicine, Dept. of Medicine, Section of Internal Medicine, University of Verona.
- **1 Oct 2023- Full professor of Medicine, Dept. of Medicine, Section of Internal Medicine, University of Verona.**

Grants and research projects

She obtained the following grants:

- **FIRB-projec**t 2001 (2003-2006)- RBNE01XHME-MIUR "Proteomic approach to erythroid cells and erythropoiesis" (PI).
- **PRIN (2004):** # 2004065788 MIUR. "Functional proteomic approach to membrane transport system in normal and diseased red cells: role of K-Cl cotransport" "(PI).
- **PRIN (2006)** # 2006063929 (MIUR): "Proteomic analysis of erythroid precursors in anemias associated with iron overload" (PI).
- **PRIN (2008): #** 2008N73CJ5 (Italian Ministry of Research): " Molecular mechanisms of hepcidin inhibition in iron deficiency, hypoxia and erythropoietic expansion: relevance to genetic and acquired iron disorders" (PI).
- **PRIN (2012)** #20128PNX83" Studio dei meccanismi molecolari dell'eritropoiesi per l'identificazione di targets terapeutici" (PI).
- **PRIN (2020)** #2020Z22PM7:" Understanding diserythropoiesis to identify new therapeutic mechanisms in hereditary anemias" (PI).
- **PRIN (2022)** # 2022E87XJW:" Understanding the role of macrophages at single cell level in sickle cell related pulmonary hypertension" (PI-coordinatrice nazionale)
- **Telethon grant GPP07007** (2007-2012) Analysis of functional and post-translational modifications in abnormal red cells from neuroacanthocytosis'. (PI).
- **Telethon grant GPP13005 (2014-2017):"** Lyn core on neuroacanthocytosis". (PI)
- **Telethon grant GPP20116 (2021-2024)**: "Identification of Druggable Pro-Resolving Mechanisms in Sickle cell Disease" (focus on SCD cardiomyopathy) (PI).
- **2008 John Grooms Wellcome Trust and the Advocacy for neuroacanthocytosis patients** (UK) project title:" Intracellular signaling in red cells from neuroacanthocytosis" (PI).
- **2012-2015 the Advocacy for neuroacanthocytosis patients Wellcome Trust** (UK) project title:" Signal transduction in red cells of patients with chorea-acanthocytosus: the Lyn connection" (PI).

- **2017-2018 the Advocacy for neuroacanthocytosis patients Wellcome Trust** (UK) Targeting the link between autophagy and Lyn to identify new therapeutic options in chorea-acanthocytosis" (PI).
- **2018-2020 the Advocacy for neuroacanthocytosis patients Wellcome Trust** (UK):" Modulation of cell signaling by targeting Lyn as New Therapeutic Options In Chorea-Acanthocytosis (PI).
- **2020-2021 the Advocacy for neuroacanthocytosis patients Wellcome Trust** (UK). Understanding the functional link between kinome and proteostasis dysfunction in chorea-acanthocytosis.(PI).
- **2020** Borsa dottorato PON-FSE-FESR (ricerca innovazione 2014-2020) (PON R&I)-azione I.1-dottorandi innovativi a carattere industriale- 36 ciclo, titolo del progetto :"Sviluppo e validazione clinica di nuove tecnologie ad elevata automazione per la diagnostica molecolare in vitro".
- **2021-2022 the Advocacy for neuroacanthocytosis patients Wellcome Trust.** Understanding the functional role of Vps13a in mouse model of chorea-acanthocytosis. (UK) (PI).

PI research grants with biopharma or private institutions (n=6)

- **2005-2010 Research grant:** Characterization of ICAs Gardos channel inhibitors *(Icagen, USA)* (PI).
- **2015-2018 Research grant** " Functional characterization of Glut 1 inhibitor in b thalassemia " (*Roche, Switzerland*) (PI).
- **2016-2020 Research grant** - "understanding the effects of new anti-sickling molecules and rADAMST13" (*Baxalta, now Takeda, Japan*) (PI).
- **2015- Research grant** "Metabolic impact of PK-activator in hemoglobinopathies and rare anemias" (*Agios, USA, **active***) (PI).
- **2021- Research grant**" Characterization of w-3 fatty acid mixture on sickle cell disease **(**Afimmune Limited, Ireland; **active**) (PI).
- **2023 Fondazione For Anemia- Convenzione-progetto di ricerca:"** A Single Center, Pilot Clinical Investigation of Safety and Efficacy of Hypoxic Red Blood Cells Manufactured with Hemanext ONE® System in Patients with β-Thalassemia Requiring Chronic Transfusion"(**active**). (PI).

Co-PI (n=4):

- 1996-1997. **Telethon grant #E491** dal titolo:" Molecular mechanisms of cell dehydration in sickle cell disease and b-thalassemia regulation of KCC1 contransport in normal and diseased erythrocytes" come co-PI.
- 1997-1998. **Telethon grant # E791** :" Molecular mechanisms of cell dehydration in sickle cell disease and b-thalassemia regulation of KCC1 contransport in normal and diseased erythrocytes" come co-PI.
- **NIH grant** (2002-2007) co-PI "red cell homeostasis and red cell magnesium content in SCA".
- Dal 2014 membro del progetto Europeo **PROTEOSTASIS (BM1307**) -**EU COST Action** (Wp3) (www.cost-proteostasis.eu).

Lucia De Franceschi is part of the teaching staff of the European School of Hematology. She is a member of the Cochrane's reviewer group on sickle cell disease. She is associated editor for the American Journal of Hematology since 2007. She is part of the editorial Board of BLOOD since 2016.

H-F ACTOR: 48 (Scopus)

Number of citation: 8206 (Scopus)

Total IF: 1860.951; # Mean IF: 9.494

 # Papers on peer-review journal with IF>12: 53 papers

Tabella Papers				
RIVISTA	**Quartile SJR 2020**	***n***	**IF Journal**	**IF totale paper(s)**
Circulation	**Q1**	**1**	39.18	39.18
BLOOD	**Q1**	**22**	25.669	564.718
J Clin Invest	**Q1**	**6**	19.456	116.736
Hepatology	**Q1**	**1**	17.298	17.298
J Thromb Haemost	**Q1**	**1**	16.04	16.04
Autophagy	**Q1**	**1**	13.391	13.391
Am J Hematology	**Q1**	**17**	13.268	225.556
Cochrane Database Syst Rev	**Q1**	**4**	12.008	48.032
Haematologica	**Q1**	**22**	11.04	242.88
Am J Gastroenterology	**Q1**	**1**	10.864	10.864
Redox Biology	**Q1**	**1**	10.787	10.787
Cell Report	**Q1**	**1**	9.995	9.995
J Clin Invest Insight	**Q1**	**1**	9.484	9.484
Liver International	**Q1**	**1**	8.754	8.754
British Journal of Haematol	**Q1**	**2**	8.61	17.22
Clin Chem Lab Med	**Q1**	**1**	8.490	8.490
Hemasphere	**Q1**	**5**	8.3	41.5
Modern Pathology	**Q1**	**1**	8.209	8.209
Free Radical Biol & Med	**Q1**	**6**	8.101	48.06
Antioxidants	**Q2**	**5**	7.675	38.375
Acta Neuropathol Comm	**Q1**	**1**	7.578	7.578
Antioxidant Redox Signaling	**Q1**	**2**	7.468	14.936

Oxidative medicine and cellular longevity	**Q1**	**2**	6.760	13.520
J Neuroscience	**Q1**	**1**	6.709	6.709
FASEB J	**Q1**	**2**	5.834	11.668
Annals of Emergency Medicine	**Q1**	**1**	5.799	5.799
J Cell Physiol	**Q1**	**1**	5.546	5.546
J Biol Chem	**Q1**	**2**	5.486	10.972
J Med Chem	**Q1**	**1**	5.484	5.848
J Cyst Fibrosis	**Q1**	**1**	5.482	5.482
J Cell Mol Med	**Q1**	**1**	5.31	5.31
Int J Obes	**Q1**	**1**	5.095	5.095
Exp Op Invest Drug	**Q1**	**1**	5.08	5.08
Frontiers in Medicine	**Q1**	**1**	5.058	5.058
J Clin Med	**Q1**	**3**	4.964	14.892
Biochem J	**Q1**	**2**	4.779	9.558
Frontiers in Physiology	**Q1**	**1**	4.755	4.755
Annals of NY Academy of Science	**Q1**	**1**	4.728	4.728
Eur J Clin Invest	**Q1**	**1**	4.686	4.686
Am J Physiol-Lung Cell Mol Physiol	**Q1**	**1**	4.659	4.659
Front Mol Biosci	**Q1**	**1**	4.62	4.62
European J of Internal Medicine	**Q2**	**4**	4.487	17.948
J Pers Med	**Q1**	**1**	4.45	4.45
Sci Report	**Q1**	**1**	4.37	4.37
J Transl Med	**Q1**	**1**	4.206	4.206
J Hypertension	**Q1**	**1**	4.171	4.171
Int J Cardiol	**Q1**	**1**	4.164	4.164
Eur J of Physiology- Pflugers Arch	**Q1**	**1**	4.084	4.084
J. of Proteomics	**Q2**	**1**	4.044	4.044
Thromb Research	**Q1**	**1**	3.994	3.994
Diagnostics	**Q1**	**1**	3.992	3.992
Sem Hematol	**Q1**	**1**	3.989	3.989
Proteomics	**Q1**	**2**	3.929	7.858
Am J Reprod Immunol	**Q1**	**1**	3.886	3.886
Seminars in Thromb and Haemost	**Q1**	**1**	3.693	3.693
Orphanet Journal of Rare Disease	**Q1**	**2**	3.687	7.374
BBA general subjects	**Q2**	**1**	3.67	3.67
Blood Transfusion	**Q2**	**3**	3.66	10.98
Med J Hematol	**Q2**	**1**	3.623	3.623
PLoS One	**Q1**	**6**	3.534	21.204
Am J Physiol (Cell Physiol)	**Q1**	**4**	3.485	13.940
Diagnostics	**Q1**	**1**	3.24	3.24
Current Opinion in Hematology	**Q1**	**3**	3.218	9.654
Pain Practice	**Q1**	**1**	3.183	3.183

Electrophoresis	**Q2**	**1**	3.161	3.161
Transfusion	**Q2**	**2**	3.157	6.314
Exp Haematol	**Q1**	**1**	3.084	3.084
Exp Physiol	**Q2**	**1**	2.969	2.969
J Pain Reserch	**Q1**	**1**	2.76	2.76
Proteomics Clinical Application	**Q2**	**2**	2.683	5.366
Vox Sanguinis	**Q2**	**1**	2.585	2.585
Eur J Gastroent & Hepatology	**Q2**	**1**	2.566	2.566
J Memb Biol	**Q2**	**1**	2.482	2.482
Minerva Pediatrica	**Q3**	**1**	2.387	2.387
Blood Cell Mol Disease	**Q2**	**3**	2.331	6,993
Drugs New Perspect	**Q2**	**1**	2.305	2.305
Acta Pediatrica	**Q2**	**1**	2.299	2.299
J Lab Clin Med	**Q3**	**1**	2.193	2.193
Epidemiol Prev	**Q3**	**1**	1.901	1.901
Med Hypothesis	**Q3**	**1**	1.375	1.375
Hemoglobin	**Q4**	**1**	1.274	1.274
Cell Mol Biol	**Q3**	**1**	1.176	1.176
Data in Brief	**Q4**	**2**	1.13	2.26
Pediatric reports	**Q3**	**1**	0.968	0.968
Sante	**Q3**	**1**	0.294	0.294
Hematologie	**Q3**	**2**	0.136	0.272
IF TOTALE		**196**		**1860.951**

PAPERS

1) Brugnara C, **De Franceschi L**. The effect of cell age and phenylhydrazine on the cation transport properties of rabbit erythrocytes. J. Cell Physiol. 154: 271-280, 1993.

2) Brugnara C, **De Franceschi L**, Alper SL. Ca^{2+}activated K^+ transport in erythrocytes: comparison of binding and transport inhibition by scorpion toxins. J. Biol. Chem. 12: 8760-8768, 1993.

3) Brugnara C, **De Franceschi L**, Alper SL. Inhibition of Ca^{2+}activated K^+ transport and cell dehydration in sickle erythrocytes by clotrimazole and other imidazole derivates. J Clin Invest. 92: 520-526, 1993.

4) Olivieri O, **De Franceschi L**, De Gironcoli M, Girelli D, Corrocher R. Potassium loss and cell dehydration of stored erythrocytes following incubation in autologous plasma: role of K/Cl cotransport system. Vox Sang. 65: 95-102, 1993.

5) **De Franceschi L,** Saadane N, Alper SL, Brugnara C, Beuzard Y. Treatment with oral clotrimazole blocks Ca^{2+}activated K^+ transport and reverses erythrocyte

dehydration in transgenic SAD mice: model for therapy of sickle cell disease. J. Clin. Invest. 93: 1670-1676, 1994.

6) Olivieri O, **De Franceschi L**, Cappellini MD, Girelli D, Corrocher R, Brugnara C. Oxidative damage and erythrocyte membrane transport abnormalities in thalassemias. Blood 84: 315-320, 1994.

7) Brugnara C, **De Franceschi L**, Armsby CC, Saadane N, Trudel M, Beuzard Y, Rittenhouse A, Rifai N, Platt O, Alper SL. A new therapeutic approach for sickle cell disease: blockade of the red cell Ca^{2+}activated K^{+} channel by clotrimazole. The Annals of the New York Academy of Science, 763:262-271, 1995.

8) **De Franceschi L,** Beuzard Y, Brugnara C. Sulphydryl oxidation and activation of red cell K/Cl cotransport in the SAD mouse, transgenic model for sickle cell anemia. Am. J. Physiol 269 (Cell Physiol: 38) C899-C906, 1995.

9) **De Franceschi L,** Olivieri O, Girelli D, Bernich P, Lupo A, Corrocher R. Red cell cation transport in uremic anemia: evidence for an increased K/Cl cotransporrt activity. Effects of dialysis and erythropoietin treatment. Eur. J. Clin. Invest. 25: 762-768, 1995.

10) Brugnara C, Armsby CC, **De Franceschi L,** Crest M, Euclaire MFM, Alper SL. Ca^{2+}activated K^{+} channels of human and rabbit erythrocytes display distinctive patterns of inhibition by venum peptide toxins. J. Membrane Biol. 147: 71-82, 1995.

11) Girelli D, Olivieri O, **De Franceschi L**, Corrocher R, Bergamaschi G, Cazzola M. A linkage between hereditary hyperferritinemia not related to iron overload and autosomal dominant congenital cataract. Brit. J. Haematol. 90: 931-935, 1995.

12) Girelli D Corrocher R, Bisceglia L, Olivieri O, **De Franceschi L**, Zelante L, Gasparini P. Molecular basis of recently described hereditary heperferritinemia-catarct syndrome: a mutation in the iron responsive element of ferritin L-subunit gene (“the Verona mutation”). Blood 86: 4050-4053, 1995

13) Russo C, Olivieri O, Girelli D, Azzini M, Stanzial AM, Guarini P, Friso S, **De Franceschi L**, Corrocher R. Omega-3 PUFA supplementation and ambulatory blood pressure monitoring parameters in mild essential hypertensive patients. J. Hypertension 13: 1823-1826, 1995.

14) **De Franceschi L**, Rouyer-Fessard P, Alper SL, Jouault H, Brugnara C, Beuzard Y. Combination therapy of clotrimazole, hydroxyurea and erythropoietin in a thalassemic mouse: a model for human therapy. Blood 87: 1186-1195, 1996.

15)**De Franceschi L**, Brugnara C, Jouault H, Beuzard Y. Modulation of erythrocyte potassium chloride cotransport, potassium content, and density by dietary magnesium intake in transgenic SAD mouse. Blood 88: 2738-2744, 1996.

16)**De Franceschi L,** Fumagalli L, Olivieri O, Corrocher R, Lowell CA, Berton G. Deficiency of Src family kinases Fgr and Hck results in activation of erythrocyte K-Cl cotransport. J. Clin. Invest. 99:220-227, 1997.

17)**De Franceschi L,** Beuzard Y, Brugnara C. Dietary magnesium supplementation ameliorates anemia in a mouse model of b- thalassemia. Blood 90: 1283-1290, 1997.

18)**De Franceschi L,** Bachir D, Galacteros F, Tchernia GJ, Cynober T, Alper SL, Platt O, Beuzard Y, Brugnara C. Oral magnesium supplements reduce erythrocyte dehydration in patients with sickle cell disease. J. Clin. Invest. 100: 1847-1852, 1997.

19)**De Franceschi L,** Olivieri O, Miraglia del Giudice E, Perrotta S, Corrocher R, Iolascon A. Anion transport and K-Cl cotransport in hereditary spherocytosis associated with different membrane defects. Am. J. Hematol. 55: 121-128, 1997.

20)Olivieri O, **De Franceschi L**, Bordin L, Manfredi M, Miraglia de Giudice E, Perrotta S, De Vito M, Guarini P, Corrocher R. Increased membrane protein phosphorylation and anion transport activity in chorea-acanthocytosis. Haematologica 82: 648-653, 1997.

21)**De Franceschi L,** Turrini F, Miraglia del Giudice E, Perrotta S, Olivieri O, Corrocher R,Mannu F, Iolascon A. Decrease band 3 anion transport activity and band 3 clusterization in congenital dyserythropoietic anemia type II. Exp. Haematol. 26: 869-873, 1998.

22)**De Franceschi L**, Cappellini MD, Olivieri O, Graziadei G, Corrocher R, Beuzard Y, Brugnara C. The effect of dietary magnesium supplementation on the cellular abnormalities of erythrocytes in patients with b-thalassemia intermedia. Haematologica 83: 118-125, 1998.

23)Vandorpe DH, Shmukler BE, Jiang L, Lim B, Maylie J, Adelman JP, **De Franceschi L**, Cappellini MD, Brugnara C, Alper SL. cDNA cloning and functional characterization of the mouse Ca^{2+}activated K^+ channel, mIK1. J. Biol. Chem. 273: 21542-21553,1998.

24)**De Franceschi L** and Beuzard Y. Red blood cell indices, cation content and membrane cation transport. Hemoglobin 22: 493-500, 1998.

25)**De Franceschi L**, Shalev O, Piga A, Collell M, Olivieri O, Corrocher R, Hebbel RP, Brugnara C. Deferipone therapy in homozygous human b-thalassemia removes erythrocyte membrane free iron reduces K-Cl cotransport activity. J. Lab. Clin. Med. 133: 64-69, 1999.

26)Su W, Shmukler BE, Chernovaa MN, Stuart-Tilley AK, **De Franceschi L**, Brugnara C, Alper SL. Mouse K-Cl cotransporter KCC1: cloning, mapping, pathological expression and functional regulation. Am. J. Physiol. 277 (Cell Physiol 46) C899-C912, 1999.

27)Beuzard Y, **De Franceschi L,** Vitoux D, Rouyer-Fessard P, Henri A. Towards therapies of hemoglobin disorders, targeting the erythrocyte. Hematologie 5: 60-75, 1999.

28)**De Franceschi L**, Brugnara C, Rouyer- Fessard P, Jouailt H, Beuzard Y. Formation of dense erythrocyte in SAD mice exposed to chronic hypoxia: evaluation of different therapeutic regimens and additive benefit of combining oral clotrimazole and magnesium therapies. Blood 94: 4307-4313,1999.

29)**De Franceschi** L, Bachir D, Galacteros F, Tchernia G, Cynober T, Neuber D, Beuzard Y, Brugnara C. Oral magnesium reduces the number of painful days in patients with sickle cell disease. Brit. J. Haematol. 108: 284-289, 2000.

30)**De Franceschi L,** Fattovich G, Turrini F, Ayl K, Brugnara C, Manzato F, Noventa F, Stanzial AM, Solero P, Corrocher R. Haemolytic anemia induced by ribavirin therapy in patients with chronic hepatitis C infection: role of membrane oxidative damage. Hepatology 31: 997-1004, 2000.

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32)Brugnara C, **De Franceschi L**, Beuzard Y. Erythrocyte- active agents and treatment of sickle cell disease. Sem. Hematol 38: 324-332, 2001.

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34)Brugnara C**, L De Franceschi,** P Bennekou, SL Alper, P Christophersen. Novel therapies for prevention of erythrocyte dehydration in sickle cell anemia. Drugs News Perspect 14: 208-220, 2001.

35)Riddington C, **De Franceschi L.** Drugs for preventing red cell dehydration in people with sickle cell disease. Cochrane Database Syst Rev 4: CD003426, 2002.

36)Stocker JW, **De Franceschi L**, McNaughton-Smith GA, Corrocher R, Beuzard Y, Brugnara C. ICA-17043, a novel Gardos channel blocker, prevents sickle red cell dehydration in vitro and in vivo in SAD mice. Blood 101: 2412-2418, 2003.

37)**De Franceschi L,** Baron A, Scarpa A, Adrie C, Janin A, Barbi S, Kister J, Rouyer-Fessard P, Corrocher R, Leboulch P, Beuzard Y. Inhaled nitric oxide protects transgenic SAD Mice from sickle cell disease-specific lung injury induced by hypoxia/reoxygenation. Blood 102:1087-1096, 2003.

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41)Favalli MN, **De Franceschi L,** Bassetto AM, Bambara LM, Mansueto G, Corrocher R. Selective intra-arterial Terlipressin infusion stops acute lower gastrointestinal bleeding: a case report and review of the literature. Eur. J. Gastroenterology & Hepatol, 16:1059-1061, 2004.

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44)**De Franceschi L,** Finco G, Vassanelli A, Zaia B, Ischia S, Corrocher R. A pilot study on the efficacy of ketorolac plus tramadol infusion in management of sickle cell pain combined with erythrocytoapheresis in treatment of acute severe vaso-occlusive crisis. Haematologica 89: 1389-1391, 2004

45)Mallozzi C, **De Franceschi L,** Brugnara C, Di Stasi AMM. Protein phosphatase 1 is tyrosine phosphorylated and inactivated by peroxynitrite in erythrocyte through the *src* family kinase *fgr.* Free Radical Biology & Medicine 38: 1625-36, 2005

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resolution of high molecular-mass proteins in 2D electrophoresis. Proteomics 5: 2331-2339, 2005

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49)Perrotta S, Borriello A, Scaloni A, **De Franceschi L**, Brunati AM, Turrini F, Nigro V, Miraglia del Giudice E, Nobili B, Conte ML, Rossi F, Iolascon A, Donella-Deana A, Zappia V, Poggi V, Anong W, Low P, Narla M, Della Ragione F. The N-Terminal 11 amino acids of human erythrocyte band 3 are critical for aldolase binding and protein phosphorylation: implications for band 3 function. Blood 106: 4359-66, 2005.

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52)Brugnara C, **De Franceschi L.** New therapeutic approaches to sickle cell disease. Hematologie 12: 239-245, 2006.

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Book chapters

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8) **Luzzatto L & De Franceschi L. Hemolytic anemias- Lucio Luzzato & Lucia De Franceschi”, chapter 100; 21st edition of Harrison’s Principles of Internal Medicine (**Marzo 2022) (allegato 2-indice-pag. 5).

Patents

1) *Canadian Patent:* no 2,308,854. Entitled: use of divalent cations for inhibiting erythrocyte dehydration in vivo.
2) *PCT:* WO2003053449A1 (WIPO) Entitled: novel use of 7-monohydroxyethylrutoside (MonoHER) for treatment of haematological disorders, particularly beta-thalassemia.
3) *PCT:* /US16/15428 entitled: Modulators of protein tyrosine phosphatase and uses thereof.

INVITED LECTURS

NATIONAL (2000-2023)

1) Lettura su invito “Ematologia non Oncologica” organizzato dal prof Sante Tura, Universita’ di Bologna (Bologna, Giugno 2000)
2) Lettura su corso ECM dal titolo: ”Emergenze in Ematologia”, Ospedale Niguarda Ca’Granda, Milano, Aprile 2003.
3) Lettura su invito:” Recent progress in thalassemia contribution by animal models and proteomic studies- SIB Riccione 28-30 settembre 2005
4) Lettura su invito: Focus sulle anemia:” Le sindromi falciformi” – Giornate ematologia vicentina III edizione 19-21 ottobre 2005
5) Lettura su invito:” Le sindromi falciformi” “convegno Sovraccarico di ferro: aspetti clinici e terapia, Milano 24-25 Maggio 2007
6) Lettura su invito “di Ematologia non Oncologica” dal prof Sante Tura, Universita’ di Bologna e prof MD Cappellini (Bologna, ottobre 2008)
7) Lettura su invito “Epidemilogia e clinica delle emoglobinopatie”. Rimini 28-31 ottobre 2008- Congresso nazionale SIBIOC- SIMeL
8) Lettura su invito corso post-laurea dal titolo" L'anemia dell'immigrato". Universita' di Bologna (organizzatori prof Tura e prof Cappellini); 21-24 Novembre 2010.
9) Relazione su invito nell'ambito delle sessioni educazionali della Societa' Italiana di Ematologia, Napoli 16-19 ottobre 2011
10) Relazione su invito “ la cura globale del soggetto affetto da emoglobinopatia”- - relazione dal titolo:” La presa in carico globale del soggetto affetto da anemia falciforme”- Milano 29-30 Aprile 2011.
11) Relazione su invito SITE 12 Novembre 2011:” Emergenze nell’anemia falciforme”.
12) Relazione su invito al corso formativo dal titolo:" Ematologia non oncologica: nuovi orizzonti" Universita' di Milano e Societa' Italiana di Ematologia; Milano 13-15 Marzo 2012
13) Relazione su invito al corso formativo:” Il sovraccarico di Ferro nelle malattie ematologiche benigne”. Relazione: ”Il sovraccarico di Ferro nell’anemia falciforme”. Monza 1 giugno 2012.
14) 27-29 settembre 2012, relazione su invito:” che rischio quella gravidanza”- congresso nazionale SITE Brindisi
15) 20 Marzo 2013, Treviso- Relazione su invito all'incontro SITE presso l'Ospedale di Treviso dal titolo:" Terzo incontro sulla Diagnostica delle Emoglobinopatie“I difetti emergenti dell’emoglobina: dal laboratorio alla clinica”;
16) 22-23 marzo 2013, Milano- Relazione su invito all'incontro dal titolo:" Ridefinire la ferrochelazione: aspetti dell'anemia falciforme
17) 12- 13 Aprile 2013, Monselice- relazione su invito all'incontro dal titolo:" la talassemia: per un approccio sistemico e multidisciplinare”.
18) 9-11 giugno Roma 2013- relazione su invio al congresso della societa' italiana di onco-ematologia pediatrica (AIEOP)- relazione dal titolo:" anemia falciforme... tutto cio' che avresti voluto sapere".
19) **Relazione su invito Societa’ Italiana di Medicina Interna (2013):” anemia falciforme problematica sanitaria emergente”.**

20) Lettura su invito: “ Lo stroke nelle sindromi falciformi” Workshop-SITE Palermo 10 ottobre 2013
21) Relazione su invito al congresso nazionale societa’ italiana per lo studio delle talassemie ed emoglobinopatie (SITE) Genova 3-5 ottobre 2014
22) Lettura su invito: “Le emoglobinopatie”- Ematologia benigna up-date Mantova 17 ottobre 2014
23) Relazione su invito alle giornate Vicentine di ematologia, :” Complicanze NON ematologiche delle emoglobinopatie” Vicenza 19-21 Novembre 2014.
24) Relazione su invito Meeting:” Red cell biology thirty years after”- relazione:” pathophysiology of sickle cell disease”. Milano 24-26 Settembre 2015
25) Relazione su invito: SIE (societa’ italiana di ematologia’ “ Complicanze emergenti dell’anemia falciforme” Firenze 4-7 ottobre 2015.
26) Relazione su invito- Meeting SIGU (societa’ italiana di genetica umana)- “Meccanismi fondamentali dell’eritropoiesi”- Rimini 21-23 ottobre 2015
27) Relazione su invito meeting Rete ematologica lombarda:” nuove terapie: drepanocitosi”- Milano 20 Aprile 2016.
28) Relazione su invito al congresso nazionale societa’ italiana per lo studio delle talassemie ed emoglobinopatie (SITE) Catania 4-7 ottobre 2016
29) “Ferro e non solo ferro- emoglobinopatie e patologie del ferro”- Cagliari 11-12 marzo 2016.
30) **SIMI-Sezione Triveneto:” Attualita’ in Medicina Interna”- Udine 24 Giugno 2016**
31) Le anemie congenite del migrante: anemia falciforme. Progetto Ematologia Romagna, Ravenna giugno 2017
32) Highlights from EHA 22-23 settembre 2017-Firenze:” novita’ EHA: I globuli rossi”
33) **SIMI- congresso nazionale. Corsi interattivi:” L’approccio del paziente con anemia in pronto soccorso”. 28 ottobre 2017**
34) Relazione su invito;’ L’anemia falciforme: nuovi approcci terapeutici”- Highlights in ematologia- Treviso 17 Novembre 2017.
35) Relazione su invito:” Drepanocitosi nuovi approcci terapeutici” – Workshop -prof Sansone-Galliera- 19 gennaio 2018.
36) Relazione su invito corso ECM:” Focus sulle emoglobinopatie , complicanze cardiologiche:” “ la drepanocitosi e le complicanze cardiovascolari”- Modena 22 giugno 2018.
37) Relazione corso residenziale Universita’ di Padova:” Introduction to translational medicine:” from bench side to besi side to the community 14-18 Gennaio 2019
38) Post_ASH_SIE:” Anemie Emolitiche”, Bologna 14-16 Febbraio 2019
39) Relazione su invito al Corso residenziale: Anemia falciforme ed emoglobinopatie nuove frontiere”, Foggia 26 ottobre 2019
40) Ematologia non maligna:” Aggiornamenti sulla sickle cell disease”- Firenze 31gennaio-1 febbraio 2020
41) Post-ASH- Novita’ dal meeting della societa’ Americana di Ematologia, Napoli 13-15 Febbraio 2020
42) Evento formative digitale:”Le talassemie dalla fisiopatologia alle prospettive di cura”. Relazione:” Complicanze delle emoglobinopatie”. 7 luglio 2021

43) **Lettura su <u>invito simposio-SIMI</u> :" Nautilus: "l'internista alla scoperta del sommerso ematologico". 23 ottobre 2021**
44) Lettura su invito: 12-13 Novembre 2021:" Problematiche ematologiche in eta' pediatrica- pratica clinica". Relazione:" Le alterazioni e le malattie renali nel bambino con drepanocitosi".
45) Post-ASH Genova- lettura su invito: "novita' nel trattamento delle anemie rare" 17-19 Febbraio 2022
46) SIMTI- relazione su invito"…non solo eritrocitoaferesi". Rimini 4-5 Aprile 2022
47) Ematologia non oncologica- relazione su invito" novita' terapeutiche nella drepanocitosi" Roma 13-14 Maggio 2022
48) Lettura su invito "Sickle cell disease: dalla patogenesi alle novita' terapeutiche" in workshop educazionale on " update sulle anemie, piastrinopenie ed emofilia" 20 settembre 2022
49) Lettura su invito -SIE Societa' italiana di ematologia :" Nuovi Approcci Farmacologici nel Trattamento dell'anemia Falciforme" 26-28 settembre 2022 Roma
50) Lettura su invito -SIE Societa' italiana di ematologia-Educazionale:" La Diagnostica delle Anemie congenite nell'adulto - 26-28 settembre 2022 Roma
51) Lettura su invito: " acute chest syndrome, atopy and bronchial herperactivity; 1 ottobre 2022- meeting Difficult allergy and asthma day.
52) Lettura su invito: Le novita' in b-talassemia: nuove prospettive terapeutiche, sicurezza ed efficacia dei nuovi farmaci- meeting BETA NETWORK Il paziente con beta-talassemia: uno sguardo oltre la terapia-accademia Medica- Ferrara, 6 ottobre 2022
53) Lettura su invito:" la gestione delle crisi vaso-occlusive nella SCD"- 21 ottobre 2022 Bolzano-meeting"L'ematologica difficile".
54) **<u>Lettura su invito:" SCD and b-thalassemia" Diagnosi difficili e malattie rare 27-28 ottobre Villa Camozzi- Bergamo- Istituto M Negri e SIMI lombardia</u>**
55) Lettura su invito:" SCD: la terapia oggi"- in Highlights in Ematologia-18-19 Novembre 2022, Treviso.
56) Lettura su invito: 15 dicembre 2022:" Problematiche ematologiche in eta' pediatrica-pratica clinica". Relazione:" Le novita' nella SCD: nuove prospettive terapeutiche, sicurezza ed efficacia dei nuovi farmaci".
57) Relazione su invito: workshop SIMTI "emoglobinopatie: supporto trasfusionale e nuovi approcci terapeutici". Relazione :" la diagnosi della drepanocitosi". Bologna, 31
gennaio 2023.

58) 8 Maggio 2023- Palermo- relazione su invito workshop:" Celebrating successes and addressing persistent unmet needs in thalassemia.:"New paradigm in the clinical management of SCD".
59) 19 Maggio 2023-Catania- relazione su invito " Drepanocitosi: update", nel meeting " la prevenzione va in porto";
60) 21-23 settembre 2023: Relazione su invito congresso nazionale SITE: "L'anemia falciforme: qualcosa è cambiato".

INTERNATIONAL (2000-2023) (n=50)

NB: Non sono riportati in elenco i seminari su invito tenuti in corsi di dottorato.

1) Lettura su invito: Scuola Europea di Ematologia (ESH): " Iron Metabolism, erythropoiesis and related disorders", Sestri Levante 15/17 October 2004.
2) Lettura su invito- societa' francese di ematologie:" Developing treatments for sickle cell disease"-Parigi, Febbraio 2004.
3) Lettura su invito European Hematology Association (EHA): "Establish and Experimental pharmacologic treatments for sickle cell disease" Stockolm June 2005
4) Lettura su invito: International workshop on NA:" Red cells in mcleod syndrome-Kyoto 27-30 ottobre 2006
5) Lettura su invito-"Phospho-signaling in red cells" International NA workshop Bethesda 1-2 October 2010
6) Relazione su invito all' "International symposium Neurological complications of Sickle Cell Disease, Padova 26 marzo 2011
7) Lecture: European Hematology Association (EHA) red cell working group: "Animal models for new therapeutic approaches in SCD" 8 June 2011
8) 6th congresso : "sickle cell disease" presso il Parlamento Europeo di Strasbourg from 12 -15.04. 2011:"Deoxygenation affects tyr phosphoproteome of red cell membrane from patients with SCD".
9) First International Symposium on Allogeneic Cellular Gene Therapy in Hemoglobinopathies; Roma 8-11 marzo 2012
10) Lettura su invito 17th Meeting of European society of neurosonology and cerebral hemodynamics.- "sickle cell disease: a clinical overview. Venice 17-19 May 2012
11) Invited lecture: NBIA meeting- " Phosphoproteomic analysis in chorea-acanthocytosis: the Lyn connection" Wein 5 February 2012
12) Lettura si invito: EHA SWG- Red cells and erythropoiesis:" Resveratrol accelerates the activation of FOXO3 and ameliorates anemia in b thalassemic mice" Stockholm 12 June 2013
13) Lettura su invito 5th European symposium on Rare Anemias- " Clinical management of hemoglobinopathies-The sickle cell syndromes" Ferrara, 15-16 Nov 2013
14) Lecture: TIF-international meeting on Thalassemia and Hemoglobinopathies. Disturbed transmebrane ion transports and sickle cell disease Abudabi 19-13 October 2013
15) Invited Lecture : workshop" Haptoglobin/hemopexin intervention in CPB-assisted cardiac surgery and sickle cell disease: rationale and clinical development perspectives- " Treatment for SCD: current and new approaches" Bern 12-14 Nov 2014
16) Invited lecture: ESH- Pathophysiology based drug treatment in SCD" Chantilly-France 23-26 April 2014.
17) Lettura su invito ESH:" SCD in adult and elderly: emerging complications and management"- Barcellona 23-24 January 2015.
18) Relazione su invito EHA-SWG Scientific meeting: Focus on Red Cell and Iron Disorders, and Myelodysplastic Syndrome (MDS)- lecture:" Emerging treatment options for SCD' ; March 6-8, 2015, Lisbon, Portugal
19) Relazione su invito EHA-SWG Scientific meeting: Focus on Red Cell and Iron

Disorders, and Myelodysplastic Syndrome (MDS)- lecture:" Clinical guidelines for diagnostics and management: Sickle cell disease- challenges in management; March 6-8, 2015, Lisbon, Portugal

20) Lecture su invito SCiF (Sickle cell international focus)- Kings College, London 15-16 June 2015
21) Lecture su invito :" Guidelines on iron overload in rare anemias"- EHA red cell working group 20 June 2016.
22) Lecture. International Society of Laboratory Hematology (ISLH); "Red cells in neuroacanthocytosis", Milano 2016
23) Congresso Societa' Americana di ematologia (ASH) ASH- meeting in San Diego Dec 2016.: Relazione su invito nella sezione "Red Cell Biology and Childhood blood disease".
24) European hematology association (EHA)- scientific working group:" Gennaio 2017: " DNA modulators and gene therapy", Barcellona, Spain.
25) 9th International meeting on neuroacanthocytosis: 23-25 March 2017 Dresden, Germany:" Impaired autophagy is combined with abnormal signal transduction in chorea-acantocytosis".
26) Invited lecture:" Red cells and oxidative stress- ESH, Paris 18-20 April 2018.
27) Invited lecture: " SCD in adult and elderly: emerging complications and management , Paris Necker SCD preceptorship" Necker, Paris 15-16 Nov 2018.
28) European hematology association (EHA)- scientific working group:" novel treatments in Sickle cell disease", Madrid 2019
29) European School of Hematology: Budapest 15-17 March 2019:" Acute sickling crisis"
30) EHA annual meeting- science in focus session:" Autophagy and terminal erythroid differentiation". 17 June 2020
31) EHA-guidelines workshop: Guideline in management of pregnancy in rare inherited anemias:" Sickle cell disease". 25 Nov 2020.
32) EHA Hematology passport 2019-2022:" relazione:"Sickle cell disease" 15 dicembre 2020
33) European School of hematology:" 5-7 March 2021:" New approaches to the management of SCD".
34) 1relazione su invito:" maouse model for ChAc" 10th International meeting on Neuroacanthocytosis Syndromes, Barcellona 12-15 Marzo 2021
35) Current challenges in hematology (C-Hem2021) 18-19 March 2021:" Clinical Management of Sickle cell disease in 2021".
36) EHA: invited lecture- Session: red cells and iron: Lecture. "Evaluation of novel fetal hemoglobin inducer drug in clinical perspective. 14-16 June 2021
37) Lettura su invito workshop:" A new era in the management of SCD: crizanlizumab". Cyprus 10 July 2021
38) Lettura su invito IASG Annual members meeting- TIF:" The care and cure of adult sickle cell disease: An overview". 21-22 October 2021
39) Lettura su invito 15th International conference on thalassemia & other hemoglobinopathies- 17th conference for patients & parents:" Sickle cell disease in adulthood" 19-21 November 2021.

40) ASCAT satelite symphosium: placeholder - Living with and managing sickle cell disease: patient and physician perspectives—Invited lecture 26 Febbraio 2022.
41) EHA meeting – Giugno 2022; relazioni su invito: Joint symposium EHA-ASH:" Olistic approach to patients wth SCD"
42) EHA meeting – Giugno 2022; relazione su invito Educational section:" Vasculopathy in SCD"
43) Global Sickle cell meeting: relazione su invito:" SCD and hemolysis"- Parigi 16-18 giugno 2022
44) Lettura su invito:"Modern management of sickle cell disease" in International symposium from genes to target therapies in hematology" 1-2 July, 2022 Firenze.
45) Lettura su invito:" Etiopathogenesis of Herpervisicosity of Red Cells and Possible Therapeutic Treatments for Sickle Cell Disorders"- Societa' Brasiliana di Ematologia- HEMO2022- 28-29 ottobre 2022
46) Relazione su invito EHA SWG red cells and iron business meeting 22 – 24 November 2022; Guidelines on gene therapy/editing in SCD.
47) Invited lecture ASH-Highlights 2022: State of the art lecture: " Treatment of sickle cell disease today: challenges and recent advantages". Lebanese Society of Hematology and Blood Transfusion" and the "Lebanese Joint Coalition Against Thrombosis"- 18 march 2023.
48) Invited Lecture – 1st symposium on innovative therapies in hematology- Talk:" New treatment options in SCD". Avellino 30-31 March 2023.
49) Invited Lecture- Science-in-Focus" Session on "Complement activation in SCD". EHA2023 meeting Frankfurt June 8-11, 2023
50) Invited Lecture- Session: "Not-so benign hematological challenges" and give a presentation on "Thrombotic risk and its management in inherited hemoglobin disorders". This session is organized in collaboration with the EHA Specialized Working Group on "Bleeding and thrombosis". EHA2023 meeting Frankfurt June 8-11, 2023.

MEETING ORGANIZATION

International Meeting

- 2014- “3 International Symposium on neuroacanthocytosis and NBIA disease”; 30 Oct-1 Nov 2014 Stresa; Italia.
- Global Sickle cell meeting- Parigi 16-18 giugno 2022

National Meeting

- -IX congresso nazionale SITE- Catania 6-8 ottobre 2016
- Meeting Del globulo Rosso & SITE- Napoli 28-29 settembre 2017
- X congresso nazionale SITE-Roma 27-29 settembre 2018
- Coordinamento della sessione educazionale “Anemia falciforme” congresso societa’ italiana di ematologia (SIE) 7-9 ottobre 2019.
- Meeting del globulo rosso &SITE- Milano 26-27 settembre 2019
- XI congresso nazionale SITE- Mod Virtual 24-26 settembre 2020
- XII Congresso nazionale SITE- Roma, 23-25 settembre 2021.

EDITORIAL BOARD MEMBER

- **2015-2020:** Membro dell’editorial board di BLOOD (IF26.6),
- **2012- ad ora:** Membro dell’advisory editorial board di Am J Hematology (IF 13.8),
- **2018- 2020** Co-Editor Frontiers in Immunology- Research Topic Signaling pathways in autoimmune neoplastic and vascular disorders (IF 8.786)
- **2015- ad ora**: Review Editor- Frontiers in drug metabolism and transport (IF 5.988)
- **2015- ad ora:** Associated Editor Frontiers in Red cell Physiology (IF 4.755)
- **2008- ad ora:** Editorial board di Blood Mol Cell Dis (IF 2.9).

REVIEWER ACTIVITIES FOR PEER-REVIEW JOURNALS

- **Blood (IF26.6)- si riporta la review activity: 42 papers**
- Lancet Hematology (IF 18.959): **1**
- **Am J Hematology (IF 13.8): si riporta la review activity: 6 papers**
- **Haematologica (IF 11.8)- si riporta la review activity: 36 papers**
- **British Journal of Haematology (IF 8.61)-si riporta review activity: 3 papers**
- HemaSphere (IF 8.3): **2 paper**
- Experimental Hematology (IF3.84): **1 paper**
- Frontiers in Frontiers in Red cell Physiology (IF 4.755): **1 paper**
- Scientific Reports (IF4.37): **1 paper**
- PlosOne (IF 3.534): **2 papers**
- Blood Mol Cell Dis (IF 2.9): **si riporta review activity: 15 papers**
- **Dal 2002- ad oggi** Membro del review panel della **Cochrane Cystic fibrosis and genetic disorders group** per problematiche terapeutiche inerenti all’anemia falciforme ed emoglobinopatie.

REVIEWER ACTIVITY IN NATIONAL AND INTERNATIONAL MEETING

- Congresso nazionale SITE: 2014, 2016, 2018, 2020
- Congresso nazionale SIE (societa' italiana di ematologia): 45°, 46°, 47°, 49° congresso
- Congresso EHA 2018
- Congresso ASH 2010 in cui ha svolto attivita' anche di coordinamento del panel dei reviewers, ASH 2023.

MEMBERSHIP

- Societa' Italiana di Medicina interna (SIMI)
- COLMED09
- Societa' italiana di ematologia (SIE)
- Societa' italiana per lo studio di emoglobinopatie e talassemie (SITE)
- European Society of clinical investigation (1994-2000)
- European Hematology Association (EHA)
- American Society of Hematology (ASH).

PI IN THE FOLLOWING CLINICAL STUDIES:

	PROTOCOL NUMBER	TITLE	PHASE	DRUG	Status
ASTRAZENECA	D5136C00008	A randomised, double-blind, double-dummy, parallel-group, multicenter, phase IIb study to evaluate the effect of ticagrelor 10 mg and 45 mg bid versus placebo in reducing the number of days with pain in young adults with Sickle Cell Disease	IIb	ticagrelor	closed
CELGENE	ACE-536-B-THAL-001	"a phase 3, double-blind, randomized, placebo-controlled, multicenter study to determine the efficacy and safety of luspatercept (ace-536) versus placebo in adults who require regular red blood cell transfusions due to beta (β)-thalassemia "	III	LUSPATERCEPT (ACE-536)	closed
CELGENE	ACE-536-LTFU-001	A Phase 3B, Open-Label, Single-Arm, Rollover Study to evaluate long-term safety in subjects who have participated in other Luspatercept (ACE-536) clinical trials	IIIb	luspatercept (ACE-536)	Ongoing
GLOBAL BLOOD THERAPEUTICS, INC.	GBT440-031	A Phase 3, Double-blind, Randomized, Placebo-controlled, Multicenter Study of GBT440 Administered Orally to Patients with Sickle Cell Disease	III	GBT440 capsules or tablets, 300 mg	closed
GLOBAL BLOOD THERAPEUTICS, INC.	GBT440-034	Studio di estensione in aperto di GBT440 somministrato per via orale a pazienti affetti da anemia falciforme che hanno partecipato a sperimentazioni cliniche di GBT440	III	GBT440 capsules or tablets, 300 mg	closed
LA JOLLA PHARMACEUTICAL COMPANY	LJ401-BT01	A MULTI-CENTER, RANDOMIZED, OPEN-LABEL, PARALLEL-GROUP STUDY with ljpc-401 for the treatment of myocardial iron overload in adult patients with transfusion-dependent beta thalassemia	II	LJPC-401	closed
NOVARTIS	CICL670AIC04	Open-label, multicenter, single arm, phase III study to collect additional safety and efficacy data with deferasirox film-coated tablets in patients completing study CICL670F2201	III	Deferasirox FCT	closed
NOVARTIS	CICL670F2201	ceritinib taken in the fasted state in adult patients with ALK	II	DEFERASIROX	closed
NOVARTIS	CSEG101A2203	A Phase II, multicenter, randomized, open label two arm study comparing the effect of crizanlizumab + standard of care to standard of care alone on renal function in sickle cell disease patients ≥ 16 years with chronic kidney disease due to sickle cell nephropathy (STEADFAST)	II	SEG101 crizanlizumab	ongoing
NOVARTIS	CSEG101A23	A phase III, Multicenter, Randomized,	III	SEG101	Clos

	01	Double-blind Study to Assess Efficacy and Safety of Two Doses of Crizanlizumab versus placebo, with or without Hydroxyurea/ Hydroxycarbamide Therapy, in Adolescent and Adult Sickle Cell Disease Patients with Vaso- Occlusive Crises (STAND) Document type: Clinical Trial Protocol EUDRACT number: 2017-001746-10 Version number: 00 (Original Protocol) Clinical Trial Phase: III Release date: 29-Jan-2019 Property of Novartis Confidential May not be used, divulged, published, or otherwise disclosed without the consent of Novartis Clinical Trial Protocol Template Version 1.0 (13-Dec-2017) This document		crizanlizumab	
NOVARTIS	CSEG101A2401B	Studio in aperto, multicentrico, di Fase IV, rollover, in pazienti con anemia falciforme che hanno completato uno studio precedente con crizanlizumab sponsorizzato da Novartis	IV	crizanlizumab	ongoing
NOVARTIS	**CSEG101AIT02-SIWORLD**		Obs	Orizanlizumab	Cancelled by Sponsor
NOVO NORDISK SPA	NN7533-4470	A multicentre trial evaluating the efficacy and safety of oral decitabine-tetrahydrouridine (NDec) in patients with sickle cell disease - ASCENT 1	II	decitabine - tetrahydrouridine (NDec)	ongoing
PFIZER	**C4061002**	**A Phase 1B, Open-Label, Multiple Dose Escalation Study to Evaluate Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of PF-07059013 in Participantswith Sickle Cell Disease**	Ib	confidential	contact
ROCHE	BO42451	A randomized double-blind phase 2a study evaluating the efficacy, safety, pharmacokinetics, and pharmacodynamics of Crovalimab as adjunct treatment in prevention of acute vaso-occlusive episodes (VOE) in sickle cell disease (SCD)	IIa	Crovalimab	ongoing
ROCHE	BO42452	A phase 1b randomized, placebo-controlled study evaluating the safety, pharmacokinetics, pharmacodynamics, and efficacy of crovalimab for the management of acute vaso-occlusive episode (VOE), in patients with sickle cell disease (SCD)	Ib	Crovalimab	ongoing

VIFOR	VIT_-763-THAL-201	A Phase 2a, Double-blind, Randomised, Placebo-controlled, Parallel Group, Multicentre Study on Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Preliminary Efficacy of Multiple Doses of VIT-2763 in Subjects with Non-transfusion Dependent Beta-thalassaemia	IIa	VIT-2763	Close

Verona, 11 November 2023

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