Curriculum Vitae

Personal information

Raffaele Cerbini

Work experience

Mar 2026 – present: Medical Officer at INPS (Italian National Social Security Institute)

Since March 2026, I serve as Medical Officer at INPS (Italian National Social Security Institute), leading institutional medical-legal assessments, disability evaluation programs, and social security healthcare governance.

2016 – present: Adjunct Professor at Sole24Ore Business School, Milan, Italy

Teaching responsibilities in the "Master in Management of Health Science, Pharmaceutical Industry and Biomed": new therapeutic scenarios in the evolution of pharmaceuticals (including Advanced Therapy Medicinal Products); medical department role and organization in pharmaceutical companies; promotional and non-promotional activities; European pharmaceutical framework legislation, deontological codes, national and supranational laws regarding bribery and corruption.

Aug 2025 – Feb 2026: Scientific Consultant for the Presidency of the Senate's Health Committee of the Italian Republic

Provided collaboration and consultancy activities to Presidency of the Senate's Health Committee of the Italian Republic on the topic of rare genetic diseases and advanced therapies (gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products). In this role I provided high-level technical and scientific expertise to the Presidency, focusing on the complex challenges of rare genetic diseases and novel therapeutic modalities.

Oct 2022 – Apr 2024: Senior Manager Scientific and Medical Affairs – Italy at Philip Morris Italy - Science Division

I have been responsible in Italy for sharing scientific assessment related to research purposes on innovative products (pursuant to Italian D.Lgs. 6/2016, which transposes the European Directive 2014/40/UE).

May 2022 – Sep 2022: Medical Director PSSR – Worldwide Medical and Safety at Pfizer S.r.l.

In this position I had the opportunity to perform medical assessment activities related to drug safety pharmacovigilance including the determination of seriousness, expectedness/listedness/labeledness, and causality of adverse events, in compliance with current regulations.

Feb 2021 – Apr 2022: Medical and Scientific Director at Pharma D&S CRO

In this position, I had the opportunity to coordinate the work of several subordinates and I was able to carry out all tasks related to the scientific direction of Pharma D&S Clinical Research Organisation (according to Italian D.M. 15 November 2011), with a strong experience in all phases of clinical trial conduct, according to ICH-GCP guidelines, from Phase I to large randomized clinical trials. I have also provided medical affairs consulting services to several pharmaceutical companies.

Feb 2020 – Dec 2020: Executive Medical Director at AveXis EU Limited (Sept 2020 onwards: Novartis Gene Therapies)

In this role I was the scientific expert for the clinical development and regulatory approval of Zolgensma® (onasemnogene aboparvovec) as gene replacement therapy for spinal muscular atrophy (SMA). This gene therapy is a groundbreaking therapy that has revolutionised the treatment of SMA because of its ability to directly target the root cause of the disease.

Sept 2018 – Sept 2019: Senior Medical Director EUMEA at Clementia Pharmaceuticals, Inc.

Reporting to the VP EUMEA/APAC I was responsible for medical affairs and clinical development in Europe, the Middle East and Africa. I worked closely with member of the regulatory team and pharmacovigilance colleagues. During this time, I had the opportunity to conduct several clinical trials in two ultra-rare bone diseases: Fibrodysplasia Ossificans Progressiva (FOP) and Multiple Osteochondromas (MO).

Aug 2016 – May 2018: Medical Director Italy, Iberia, Greece, Eastern European Countries, Israel at BioMarin Europe Ltd.

Medical Director, member of management team, appointed by company as Qualified Person for Scientific Service in AIFA. I provided medical support for the company's product development strategy and post-marketing commitments, while supporting the global medical affairs strategy. I represented the medical division, contributing to the area's strategic medical and business plan.

I also provided scientific input (including efficacy analysis and safety signal detection with *ad hoc* risk mitigation plan) for gene therapy in haemophilia A (AAV-factor VIII vector, valoctocogene roxaparvovec), and disseminated medical awareness related to treatment with enzyme replacement therapies (e.g.: intracerebral administration of cerliponase alfa for Batten disease, appropriate treatment strategies for several mucopolysaccharidosis, etc.) while being European final medical signatory for treatment of phenylketonuria.

Jan 2013 – June 2015: Medical Director Italy at The Medicines Company (Italy) Srl

Medical Director, member of management team, appointed by company as Qualified Person for Scientific Service in AIFA. I worked closely with the international cardiovascular team, participating in discussions on clinical data for new compounds to be submitted to the EMA for approval. I have assisted in the design, conduct and publication of a major clinical trial (Matrix), which recruited over 6000 patients and whose results are currently one of the

cornerstones of interventional therapy in patients with acute myocardial infarction

Sep 2008 – Nov 2012: Field Medical Mgr to Medical Director Greece to CV/Metabolics Lead at Bristol-Myers Squibb Italy and Greece

Managed a group of twelve people in multidisciplinary teams (medical – marketing – sales) in commercial alliances with AstraZeneca and Pfizer, leading and managing medical education programmes (e.g. CME courses, publication plans, observational studies, etc). I led the medical support for the launch of apixaban in patients with nonvalvular atrial fibrillation, overseeing large randomised clinical trials in Italy.

Jan 2006 – Jul 2007: Medical Manager at Abbott Italy

I provided medical expertise and contributed to the design of international clinical trials, providing expertise in the evaluation of new compounds. I coordinated national scientific advisory boards to support to launch of adalimumab (monoclonal antibody binding to TNF-alfa) in the approved and upcoming indications.

Mar 2005 – Oct 2005: Field Medical Manager at Amgen Italy

I provided medical expertise on compounds in development and liaised with key opinion leaders across Italy.

Oct 2002 – Dec 2004: University Researcher at University of Perugia

Clinical researcher in internal and emergency medicine with extensive experience in thrombosis and haemostasis. Hospital experience in the management of complex patients with cardiovascular diseases. Research projects with publications in national scientific journals.

Education and training

2023–2024: **Management training course for General Managers** of local health authorities, hospitals and other entities. Scuola Umbra di Amministrazione Pubblica. Perugia, Italy (Umbria Region – Determinazione Dirigenziale n. 3783 on 10 April 2024; Umbria Region – Determinazione Dirigenziale n. 721 on 24 January 2025).

2009: **Internship.** UCLA Anderson Business School. Los Angeles, CA (USA).

2009: **Executive Master in Business Administration**. SDA Bocconi School of Management, Milan, Italy.

2002: **Specialization (PhD) in Internal and Emergency Medicine**. University of Perugia, Italy.

2001: **Master in Phlebolymphology**. University of San Marino, Republic of San Marino.

1996: **Master's degree in Medicine and Surgery**. University of Perugia, Italy.

Additional information

Publications

Regarding the request to provide a list of publications, please note that my primary contribution to scientific research has been focused upstream within the pharmaceutical and biotechnology sectors—specifically on the strategic design, protocol development, and methodological architecture of major global multicenter clinical trials. In accordance with industry standards and international governance guidelines (such as *Good Publication Practice* – GPP), authorship of primary clinical trial manuscripts is attributed to the Principal Investigators and clinical researchers who actively managed trial sites, enrolled patients, and gathered clinical data, rather than the sponsor-side medical strategists who conceived and designed the studies. To demonstrate my direct intellectual contribution to the generation of this clinical literature, below is a representative list of key international clinical trials to whose protocol conception and realization I directly contributed, complete with their ClinicalTrials.gov identifiers and principal publications.

- **STR1VE-EU** (*Single-Dose Gene Replacement Therapy Clinical Trial for Participants With Spinal Muscular Atrophy Type 1*) — ClinicalTrials.gov ID: **NCT03461289** | Published in: Mercuri et al. *Lancet Neurol* 2021;20(10):832-841.
- **BMN 270-301** (*Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients*) — ClinicalTrials.gov ID: **NCT03370913** | Published in: Ozelo MC, et al. *N Engl J Med* 2022;386(11):1013-1025.
- **GENEr8-2** (*Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at a Dose of 4E13 vg/kg*) — ClinicalTrials.gov ID: **NCT03392974**.
- **AVERROES** (*A Phase III Study of Apixaban in Patients With Atrial Fibrillation*) — ClinicalTrials.gov ID: **NCT00496769** | Published in: Connolly SJ, et al. *N Engl J Med* 2011;364:806-817.
- **ARISTOTLE** (*Apixaban for the Prevention of Stroke in Subjects With Atrial Fibrillation*) — ClinicalTrials.gov ID: **NCT00412984** | Published in: Granger CB, et al. *N Engl J Med* 2011;365:981-992.

Projects

Dec 2025 – Current: Vice-President, Health Promotion Council at Municipality of Perugia, Italy
The Health Promotion Council is a proposal, consultative, and support body for the municipality administration. It monitors the needs of citizens and the health and socio-health services, as well as affiliated healthcare facilities. It verifies the programmatic management guidelines, the status of implementation, and the correspondence between results and the objectives established by the Health General Management. The Council fulfils the proposal-making, consultative and participatory duties.

Oct 2025 – present: Regional President, AMICI ETS (Patient Organization) – Umbria at AMICI ETS (Italian National Association for Inflammatory Bowel Diseases)
AMICI ETS mission is to safeguard the health, rights and quality of life of individuals living with IBD, including a substantial paediatric population,
through advocacy, patient support, scientific education and research. Our governance structure is democratic and transparent, with elected bodies at national and regional levels and full public disclosure of financial and operational reports, ensuring accountability and authentic representation of patient interests.

Memberships

Editorial board member of Official Journal at Physicians, Surgeons and Dentists Professional Society of Perugia, Italy.

- Cerbini R, Solinas A. PDTA MICI Umbria 2025: un patto medico-paziente per una sanità integrata, misurabile e umana. Bollettino dell'Ordine dei Medici Chirurghi e Odontoiatri della Provincia di Perugia. 2025; 1 (editorial).
- Cerbini R. Malattie Rare: una sfida globale, una speranza concreta. Bollettino dell'Ordine dei Medici Chirurghi e Odontoiatri della Provincia di Perugia. 2025: 2; 31-33.
- Arcioni F, Cerbini R. La terapia di editing genomico per le emoglobinopatie. Bollettino dell'Ordine dei Medici Chirurghi e Odontoiatri della Provincia di Perugia. 2025: 3; 24-27.

Lecture - Cerbini R. L'importanza strategica delle terapie avanzate. Comunicazione nel convegno "Il primato italiano

nel trattamento delle emoglobinopatie". Roma, Sala dell'Istituto di Santa Maria in Aquiro, 10 Giugno 2025.

Other Relevant Information	I am a patient representative