

Serena Gasperini

PERSONAL INFORMATION

 Fondazione IRCCS-San Gerardo dei Tintori



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Sex Female | Date of birth

| Nationality Italian

JOB APPLIED FOR POSITION

Medical Doctor, Paediatricians

WORK EXPERIENCE:

I am a Resident physician, in charge at the Metabolic Unit-Paediatric Clinic ASST-Monza San Gerardo Hospital in Monza.

I work with a metabolic expert team including nursing, secretary, data manager, dieticians. We take care of many patients with metabolic diseases: lysosomal storage diseases (MPS, Pompe disease, Fabry, Gaucher, Niemann Pick), Urea cycle disorders and other aminoacidopathies, organic acidurias, fatty acids defects and glycogen storage diseases, glycosylation defects. I am also involved in newborn screening in Monza since 2015. I have a large experience in diagnosis, treatment and follow-up of patients diagnosed by newborn screening for my past experience in Florence at Meyer Children Hospital where I attended Paediatric specialization and I worked as physician in Metabolic and Neuromuscular disorders Unit and in Pediatric Neurology Department since 2004 till 2011.

During last 20 years I acquired expertise in many metabolic and neurometabolic disorders, in laboratory and molecular assay, in clinical trials.

When I worked in Meyer Hospital in Tuscany, I acquired a great experience in expanded newborn screening by tandem mass spectrometry: the pilot program to detect about 40 metabolic diseases started in 2002 in Tuscany, as the first region in Italy.

I participated to many clinical trials (especially for lysosomal storage disorders) and many of them are still going.

In particular I am principal investigators for intrathecal Study for MPSIIIA Phase IIb (HGT-SAN-093 and SHP-610-201) and for Orphazyme study Phase II/III on Niemann Pick type C (NPC-002), Phase III double blind randomized study on pegzilarginase on patients affected by Argininemia type 1. I attend to many Registries: Pompe, Fabry (FOS), Hunter (HOS), MPSVI Tyrosinemia type I. I collaborate with Fondazione Tettamanti for research on Bone alterations on mice affected by MPSI and with TIGET of San Raffaele Hospital in Milano for next gene

therapy in MPSI patients. The list of trials and ICH-GCP Experience are attached.

QUALIFICATIONS		
Qualification	Date	Granting Organisation
1. MD degree	20 October 1994	<i>University of Florence</i>
2. Licence for Medical Practice	May 1995	<i>Ministry of Health</i>
3. Diploma of Territorial First Aid Medicine (D.E.U.)	December 1996	<i>U.S.L. 3 Hospital of Pistoia</i>
4. Diploma of Pediatrics	24 October 2000	<i>University of Florence</i>
5. Diploma of Neonatal Echography	2003	<i>Rome</i>
8. Diploma of pediatric advanced life support (PALS)	2011	<i>Italian Resuscitation Council and University of Milano Bicocca</i>
9. University Master course concerning Rare pediatric diseases: methodologies for assistance, learning and research	2010	<i>University of Florence - Department for Women and Childrens' Health</i>
10. Medical registration: Ordine dei Medici di Pistoia	1995	n° 1557

PRESENT POSITION

POSITION	Resident physician at the Metabolic Unit-Paediatic Clinic ASST-Monza San Gerardo Hospital Monza-Italy
ORGANISATION	ASST-Monza San Gerardo Hospital- Fondazione MBBM
FULL ADDRESS	METABOLIC RARE DISEASES UNIT – Paediatic Clinic Fondazione MBBM ASST-Monza San Gerardo Hospital Via Pergolesi, 33, 20900 Monza-Italy
START DATE	Since July 2011

PAST POSITIONS HELD

Position	Date (from-to)	Institution/Organisation
1. Resident physician at the Ereditary Metabolic Unit and Neuro- muscular disorders (including laboratory, regional screening centre for hyperthyroidism and metabolic diseases, day hospital and hospitalization department)	From 16 January 2004 to 15 July 2011	<i>Azienda Ospedaliero-Universitaria Meyer of Florence, Metabolic and Neurology Unit</i>
2. Permanent Hospital position at the Pediatric Operative Unit	From 1 November 2002 to 15 January 2004	<i>Hospital of Valdichiana (Siena)</i>

3. Temporary Hospital position at the Pediatric Operative Unit	From 16 April to 30 September 2002	Hospital Concern of Valdichiana (Siena)
4. Consultant at the Operative Unit of the Pediatric Department I (Metabolic and Neuromuscular rare disorders Unit)	From 26 March 2001 to 31 March 2002	Azienda Ospedaliero-Universitaria Meyer of Firenze
5. Fellowship at Neonatal Intensive Care Department	From year 1997	Azienda Ospedaliera Careggi of Florence
6. Fellowship at Pediatrics Department I and Neurometabolic Diseases laboratory	From year 1996	Azienda Ospedaliero-Universitaria Meyer of Florence
7. Residency at the Pediatrics Department I	1995-1996	Azienda Ospedaliero-Universitaria Meyer of Florence
8. Post-lauream apprenticeship	November 1994- May 1995	Azienda Ospedaliera Careggi of Florence

Mother tongue(s) ITALIANO

Other language(s)	UNDERSTANDING		SPEAKING		WRITING
	Listening	Reading	Spoken interaction	Spoken production	
English	B1	B2	B1	B2	B2

Levels: A1/A2: Basic user - B1/B2: Independent user - C1/C2 Proficient user
[Common European Framework of Reference for Languages](#)

Communication skills Good communication skills gained through my experience in Hospital with parents (counselling) and children; volunteer experience in emergency unit

Organisational / managerial skills ▪ Good competence of team-leading acquired during my experience in emergency situations and in coordination of multidisciplinary team in Hospital

ICH-GCP Experience		
ICH-GCP BASIC COURSE	Giugno 2021	
Therapeutic Area/Indication	Date (from-to)	Notes
1. Glycogen storage disease type II (Pompe disease)	2004	For therapeutic trial with MYOZYME (Enzymatic replacement therapy with acid maltase)
2. Glycogen storage disease type II (Pompe disease)	2006-2008	Multicenter project for Italian Guidelines
3. A multi-center open label extension study of HGT-1111 (recombinant human arylsulfatase A) treatment of patients with Late Infantile Metachromatic Leukodystrophy – Shire HGT-MLD-049	2009-2010	International multicenter clinical trial for Shire HGT, Inc.

study		
4. A multi-center study of ERT in MORQUIO Syndrome (MPS IV)	2010	International multicenter clinical trial for BIOMARIN
5. A randomized, controlled, open label, multicenter, Phase IIb for safety and efficacy of HGT-1410 administration via an intratecal drug delivery in Pediatric patients with MPS IIIA (HGT-SAN-093)	2015-2016	International trial Phase IIb, sponsored by Shire
6. An open label Extension study Phase IIb for safety and efficacy of HGT-1410 administration via an intratecal drug delivery in Pediatric patients with MPS IIIA (SHP-610-201)	2016	International trial Phase IIb, extension study sponsored by Shire
7. Phase II/III study on arimocvlomol (heat shock protein) on Niemann Pick Type C patients	2015-2017	International multicenter clinical trial of ORPHAZYME
8. Phase III randomized-double blind PEACE study CAEB1102-300A on Argininemia patients	2020-2022	International multicenter clinical trial of AEGLEA BioTherapeutics Inc.
9. Long-Term Management of organic Acidemia Patients with CARBAGLU®	2020-ongoing	International observational study (PROTECT trial) by Recordati Rare Disease

Digital competence

SELF-ASSESSMENT

Information processing	Communication	Content creation	Safety	Problem solving
Independent user	Independent user	Independent user	Independent user	Independent user

Levels: Basic user - Independent user - Proficient user
[Digital competences - Self-assessment grid](#)

Driving licence

A and B

ADDITIONAL INFORMATION

I am a member of Italian scientific society as Società Italiana di Pediatria (SIP) and Società italiana di malattie metaboliche ereditarie e screening neonatale SIMMESN oltre che del "Society for the Study of Inborn Errors of Metabolism (SIMMESN).

Autorizzo il trattamento dei miei dati personali ai sensi del Dlgs 196 del 30 giugno 2003 e dell'art. 13 GDPR.

Monza, June 5th 2023

Serena Gasperini