PERSONAL INFORMATION

Serena Gasperini

Fondazione IRCCS San Gerardo dei Tintori
Via Pergolesi 33-20900 Monza

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 Rare disease Unit-Pediatric Department, Fondazione IRCCS San Gerardo dei Tintori in Monza.
I work with a metabolic expert team including nurses, secretary, data manager, dieticians and psychologist.
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 expanded newborn screening in Monza since 2015 as one of the 3 Clinical centre in Lombardia. 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 Pediatric specialization and I worked as physician in Metabolic and Neuromuscular disorders Unit and in Pediatric Neurology Department since 2004 till 2011.
During last 25 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.
Recently we are involved in a new trial of Phase 3 on Infantile Onset Pompe disease (IOPD) called BabyCOMET as the second national centre in Italy and ten around the world. Two other clinical trials are undere evaluation of Ethical Committee.
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.

I participated to many Advisory Board and national and international meeting as speaker or preceptorship at Master.
Since 2016 I am the reference of the Health care provider San Gerardo di Monza for the MetabERN (European Reference Network). Recently we obtained the accreditation for the satisfactory evaluation results of European Commission.
Since 2017 I was Head of Unit of Metabolic Rare Disease Unit in Monza.

EDUCATION AND TRAINING

	Qualification	Date	Granting Organisation
1.	MD degree	20 October 1994	*University of Florence*
2.	Licence for Medical Practice	May 1995	*Ministry of Health*
3.	Diploma of Territorial First Aid Medicine (D.E.U.)	December 1996	*U.S.L. 3 Hospital of Pistoia*
4.	Diploma of Pediatrics	24 October 2000	*University of Florence*
5.	Diploma of Neonatal Echography	2003	*Rome*
6.	Diploma of pediatric advanced life suppport (PALS)	2021	*American Heart Resuscitation*
7.	University Master course concerning Rare pediatric diseases: methodologies for assistance, learning and research	2010	*University of Florence - Department for Women and Childrens' Health*

8. **Medical registration: Ordine dei Medicidi Pistoia** **1995** **n° 1557**

PRESENT POSITION

POSITION	Resident physician at the Metabolic Unit of rare diseases-Pediatric Department, Fondazione IRCCS San Gerardo dei Tintori Monza-Italy
ORGANISATION	Fondazione IRCCS San Gerardo dei Tintori
FULL ADDRESS	METABOLIC UNIT of RARE DISEASES - Paediatric Department Fondazione IRCCS San Gerardo dei Tintori Via Pergolesi, 32 20900 Monza-Italy
START DATE	Since 16 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	*Azienda Ospedaliero-Universitaria Meyer of Florence, Metabolic and Neurology Unit*
2. Permanent Hospital position at the Pediatric Operative Unit	From 1 November 2002 to 15 January 2004	*Hospital of Valdichiana (Siena)*

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 multidiciplinary team in Hospital

ICH-GCP Experience

ICH-GCP BASIC COURSE	**Giugno 2023**	
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
10. Clinical Study for Treatment-naïve IOPD Babies to Evaluate Efficacy and Safety of ERT with Avalglucosidase Alfa **(Baby-COMET)**	2022-ongoing	Phase 3 ClinicalTrials.gov Identifier: NCT04910776 by Sanofi Genzyme

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 **SIMMES**N. I am also a member of international "Society for the Study of Inborn Errors of Metabolism (**SSIEM**)" and **MetabERN** (European Reference Network in Metabolic Disorders).

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Autorizzo il trattamento dei miei dati personali qui riportati in accordo al D. Lgs. n.196/2003 ai sensi dell'art. 13 D. Lgs. 30 giugno 2003 n°196 – "Codice in materia di protezione dei dati personali" e dell'art. 13 GDPR 679/16 – "Regolamento europeo sulla protezione dei dati personali".

Le dichiarazioni rese nel presente curriculum sono sa ritenersi rilasciate ai sensi degli artt. 46 e 47 del DPR 445/2000.

Monza, December 20th 2023 **Serena Gasperini**