SYNOPSIS – NB-SCI protocol v 2.3 , 05/04/2023

Study title
Prospective Study Registry regarding peripheral neuroblastic tumours presenting with spinal canal involvement (SCI)

Sponsor
Associazione Italiana Ematologia-Oncologia Pediatrica (**AIEOP)**, Italy

Coordinating Center
IRCCS Istituto Giannina Gaslini, Genova, Italy

Principal Investigator
Shifra Ash, Petach-Tikva, Israel

Co-Principal Investigator
Stefania Sorrentino, Genova, Italy
Toby Trahair, Sydney, Australia

Partecipating centres
Paediatric Haematology-Oncology Units of several European and extra-European Countries

Time schedule
First patient enrolment: May 2014
Last patient enrolment: May 2029

Background
Approximately 15% of peripheral neuroblastic tumour patients present with extradural SCI, of whom ~50% are symptomatic. The common treatment options are neurosurgical decompression, chemotherapy, and radiation therapy, but the optimal approach remains undefined. In addition, the majority of these patients develop significant long-term morbidities.

Study design
Multi-centre, observational, prospective Study Registry

Study population
Patients in the age range 0-18 years diagnosed with peripheral neuroblastic tumour and SCI in the period from May 2014 to May 2029.

Primary objective
To describe natural history of peripheral neuroblastic tumour presenting with SCI and to evaluate the combined effects of different risk factors on final neurological and orthopedic outcomes.

Secondary objectives
a) To correlate pathological and biological characteristics with clinical features, response to therapy and sequelae
b) To share the diagnostic and therapeutic approaches adopted in the participating Centres
c) To increase the communication regarding peripheral neuroblastic tumour patients presenting with SCI
d) To develop common guidelines for the management of children with any peripheral neuroblastic tumour presenting with SCI

Inclusion criteria
a) Diagnosis of peripheral neuroblastic tumour – PNT (neuroblastoma, ganglioneuroblastoma, ganglioneuroma)
b) Symptoms or imaging of SCI
c) Age <18 years
d) minimal planned follow-up of 5 years
e) parent/patient informed consent

Exclusion criteria
Invasion of intervertebral foramina only

Patient data to be collected and included in the corresponding Forms
a) work-up at diagnosis, including radiology report (CT or MRI)
b) treatment administered
c) response to treatment, including radiology report
d) follow-up
e) outcome

Good Clinical Practice Conformity
The study will be performed according to the international accepted guidelines of Good Clinical Practice (ICH–E6(R2), of Dec 15 2016) including archival storage of essential documents.

Ethical aspects
Patient registration requires an informed written consent approved by the Local Ethical approved by the local Ethical Committee.

Funding This is a collaborative clinical study which will be carried out in kind by the participating institutions. No payments will be made for participation in this Prospective Study Registry.