Newsletter

GABA-1 CLINICAL TRIAL

Issue 3 | October 2018

1st GABA-1 patient enrolled!

Congratulations to the site of Erlangen for officially starting the study!

Dear Study Members,
we are very happy to release and share with you the 3rd issue of the "GABA-1 clinical trial newsletter", containing these amazing news.

During the last month, the German site of Erlangen activated the GABA-1 study, randomizing the first two patients!
A great after-holidays start!

With the current issue we want to give to the collaborators involved in the GABA-1 study, an overview on:

- regulatory status - agreements – sites activation – readiness to patients' enrolment;
- activities promoted by the sponsor to overcome the issues identified among the active clinical sites;
- suggestions for clinical staff.

GABA-1 Highlights

The Erlangen site has **randomized 2 patients**, currently performing the stage 2 of the protocol.

Abiogen Pharma has released a **new batch of IMP visit kits**, that replace the ones currently stored at the active sites, expired on the 30th of September.

Dompè Farmaceutici's **non clinical study** requested by German CA to allow the recruitment of patients from 3 months to 3 years of age is ongoing and will be completed by the end of October.

The GABA-1 protocol is currently **fully approved** in 6 Countries: Albania, France, Germany, Greece, Italy and The Netherlands.

8 centres are **active** and performing **patients' screening.**

Other 2 centres within these Countries will soon perform SIVs for a total of 10 **recruiting centres**.

The full **regulatory approval** of GABA-1 in Poland and the UK is expected by the end of October.

PHARM srl is currently recruiting a **new CRA** for taking over the monitoring and supporting activities among the French sites.
Until this new collaborator is operational, we highly encourange the local clinical staff to contact the Sponsor for any support they might need.

GABA-1

Regulatory status - Agreements – SIVs – Enrollment readiness

Country	Clinical Site	Protocol approval	Agreem. Status	SIV performed	Enrollment status
Albania	Qendra Spitalore Univ Nene Tereza - Tiranë	Approved	Under finalization	X	Actively screening
France	H. Robert Debré - Paris	Approved	Signed	✓	Patients enrolled
	H. Necker - Paris		Signed	✓	Patients enrolled
	H. La Timone - Marseille		Signed	X	Almost ready for enrollment
	Centre H. Régional Universitaire de Lille		Signed	✓	Patients enrolled
Germany	Univ. Klin. Erlangen	Approved	Signed	✓	Still work to do
Greece	Genicom Nosokomeio Paidon I Agia Sofia	Approved	Signed	✓	Actively screening
Italy	Az. Osp. – Univ. Consorziale Policlinico di Bari	Approved	Signed	✓	Patients enrolled
	Istituto Giannina Gaslini – Genova		Under finalization	✓	Almost ready for enrollment
Netherlands	Erasmus Universitair Medisch Centrum Rotterdam	Approved	Signed	✓	Actively screening
	University Medical Center Utrecht		Under finalization	X	Almost ready for enrollment
Poland	Children's Memorial Health Institute Warsaw	Waiting for approval	Signed	X	Almost ready for enrollment
UK	Alder Hey Children's Hospital Liverpool	Waiting for approval	Under finalization	X	Still work to do

Approved · Waiting for approval · Under finalization · Signed · ✓ Yes · X No

Patients enrolled · Actively screening · Almost ready for enrollment · Still work to do

COMMUNICATION

❖ GABA-1 Meeting

A one-day meeting will be organized involving the GABA-1 Investigators from each participating Center and the Members of the DSMC (Data and Safety Monitoring Committee) in order to:

- Discuss about protocol's amendment and possibly review its main points, including Inclusion/Exclusion Criteria;
- Find feasible alternatives to overcome problems/obstacles and speed up the process of patients enrollment

Two different meeting are planned: one with Investigators and DSMC and one ad-hoc meeting reserved to the DSMC only.

More details about the meeting's proposed location and date will be circulated in the next weeks.

In preparation of the meeting's discussion, a check list has been prepared by the Sponsor and sent to the PIs in order to start collecting inputs about the issues encountered so far and related to specific Inclusion/Exclusion Criteria. This will be used to start evaluating their impact on patients' enrollment.
All the sites's personnel is invited to complete it and share it.

COMMUNICATION

❖ Destruction of expiring IMP visit kits at the clinical sites and re-supply

The old batch of GABA-1 IMP visit kits expired on the 30th of September.

During the last month, the Sponsor has coordinated with the sites' pharmacies and managed the central/local disposal of the expiring kits, and the re-supply of the newly-released batches.

Pharmacists that have not done it yet, are asked to send the information and documentation requested by the Sponsor's Drug Manager in order to comply to the GABA-1 Specific Procedures and to speed up the process.

❖ Monthly Teleconference with GABA-1 Cinical Staff

A dedicated monthly teleconference is performed **every last Friday of the month** to:

- keep the staff of all the sites and the Sponsor **updated about the progress** of the study,
- **discuss hot topics** with GABA-1 clinical staff.

At the beginning of the enrollment phase of the GABA-1 study, this monthly appointment is a **valuable occasion** to share and discuss every concern or issue arisen, to find better strategies together.

PHARM strongly encourages all the collaborators involved in the GABA-1 study at each site to put every effort to participate to the TC.

Some suggestions for the clinical staff:

Local destruction of IMP visit kits

Sites' pharmacies should destroy the expiring GABA-1 visit kits by organizing local disposal (or centralized, where no possible otherwise). Staff must follow the local procedures but also the **GABA-1 SOPs for** both **disposal and resupply of IMPs**, and in particular the most recent version of the **SSP.03 "IMPs' Supply, Dispensing and Handling_V4.0**". If you do not have it yet, please ask a copy to the Sponsor.

Do you need to practice with the eCRF?

Follow the link to the **DEMO version**:
https://pilot.advicepharma.com/demo_gaba1/login
and use these credentials:
username: test_pi password: test_pi01
Don't forget to have the **eCRF user manual** at hand, and the useful "**GABA-1 eCRF_Guide for randomization&dispensation**", recently sent by email to the sites' staff by our Data Manager.

Ready for the FIRST patient?

It is always very useful to have a read at the **GABA-1 study manual** and the study **SOPs**, to have a practical overview of the operations to be performed during the study.
Ask the Sponsor's Clinical Operation Manager to make sure that you have the most updated versions of these important documents at hand.

Are you trained for the C-SSRS?

1. All the Investigators should **Register** at:
https://secure.trainingcampus.net/uas/modules/trees/windex.aspx?rx=c-ssrs.trainingcampus.net
2. Activate and log into your new account
3. Enter «My Activities» section and select the course in **your local language**
4. Attend the course and review the case studies to get your **C-SSRS** training certificate!

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