

Mid-term Recruitment Report (Month 30)

Quarterly report V1.0

Publishable Summary

This quarterly report provides information on the project progress within the AMYPAD Prognostic and Natural History Study (WP4), with focus on the period between December 2018 and March 2019. The report is fully data-driven and focuses on submitting and obtaining approvals (ethical and regulatory) for the study protocol in all sites, site activation, and subject recruitment.

To account for the slow recruitment into EPAD, this report also describes the current efforts and plans for the inclusion of non-EPAD cohorts as parent cohorts for the PNHS. In short, any non-EPAD cohort will only become eligible after extensive evaluation of common variables across studies, compatible population characteristics and original objectives of the study. In addition, separate Collaboration Agreements will be established with these cohorts to govern data sharing and use, as well as potential IP issues. So far, at least 6 cohorts have been identified from 4 partners involved, and active recruitment from at least one of those will commence in May.

This report highlights the efforts of the Sponsor and WP leads to put in place the frame required to reach the study's objectives. This effort was directed at supporting sites in their protocol submission process, as well as to increasing the recruitment in already approved sites.

This document starts with a brief summary of the status at the end of the past period (up to and until IMI Midterm Review) and provides a detailed data-driven report on progress between the months of December 2018 and March 2019, with key performance metrics such as the numbers of eligible participants, recruitment status per active site, individual site activation and performance, selection criteria for subject selection and details of non-EPAD cohorts under consideration.

Since the previous period, the AMYPAD PNHS went from having one active site (VUmc) with 14 subjects accepting to participate and 1 scan performed, to having three active sites (VUmc, UEDIN, BBRC) and 67 acceptances to join the study, of which 47 have been formally consented and 47 scans have been performed. In addition, two more sites (UNIGE and CHUT) have received all approvals and will be activated mid-April. Projections for 2019 indicate the PNHS should have at least 640 subjects enrolled by the end of 2019, from a combination of EPAD LCS and non-EPAD cohorts.

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Further, AMYPAD PNHS has recognized the need to engage additional ongoing cohorts from which to recruit, as the availability of EPAD LCS subjects is lower than expected at the project start. A number of potential cohorts have been approached and the details of their activation are being finalized in the upcoming quarter. By including non-EPAD cohorts, the number of eligible participants for enrolment into PNHS goes from 1.197 (current EPAD-LCS numbers) to at least 2.632. This will ensure AMYPAD PNHS has a sufficient pool of participants to enrol up to 2,000 subjects, considering a 25% refusal rate.

To conclude, several mitigation plans have already been put in place to ensure the success of the project, and the WP4 team is closely monitoring progress to be able to identify further risks and resolve them in a timely manner. Overall, there is a tangible progress rate in the PNHS since first approvals at VUmc and the expectation is that by the end of the year, the study is back on track and able to reach its final goal successfully.

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