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Evaluation of the Effect of Lomitapide Treatment on Major Adverse Cardiovascular Events (MACE) in Patients with Homozygous Familial Hypercholesterolemia: Study Protocol of the LILITH Study

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ABSTRACT

Homozygous Familial Hypercholesterolemia (HoFH) is a rare genetic disorder characterized by markedly elevated low-density lipoprotein cholesterol (LDL-C) levels from birth, leading to premature and severe cardiovascular disease. Lomitapide, an inhibitor of microsomal triglyceride transfer protein (MTP), effectively lowers LDL-C in HoFH patients. However, data on its impact on major adverse cardiovascular events (MACE) remain limited, and randomized controlled trials are not feasible due to the rarity of the condition and ethical constraints. This article presents the protocol of the LILITH study (Evaluation of the Effect of Lomitapide Treatment on Major Adverse Cardiovascular Events in Patients with Homozygous Familial Hypercholesterolemia), a multicenter, observational, retrospective–prospective cohort study. The study aims to compare the incidence of MACE during the first three years of lomitapide treatment with that observed in the three years preceding treatment, within the same cohort of adult HoFH patients (target N=72). Clinical data, including MACE, lipid levels, liver function, safety outcomes, and concomitant lipid-lowering therapies, will be collected. The primary analysis will apply McNemar's test to assess changes in MACE incidence pre- and post-treatment. This methodological approach enables the evaluation of long-term cardiovascular outcomes in a real-world setting for a rare disease population.

Keywords
Homozygous Familial Hypercholesterolemia; Lomitapide; Methodology; Observational Study; Major Adverse Cardiovascular Events; Rare Disease

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Introduction

Homozygous Familial Hypercholesterolemia (HoFH) is a rare and life-threatening genetic disorder characterized by extremely elevated plasma levels of low-density lipoprotein cholesterol (LDL-C) from birth. The chronic and severe LDL-C burden leads to accelerated atherosclerosis and early-onset cardiovascular disease, often manifesting within the first two decades of life, with a significant impact on life expectancy and quality of life [1-5]. In light of this high-risk profile, the recently published international guidelines underscore the importance of early diagnosis and the attainment of ambitious LDL-C targets (<1.4 mmol/L or even <1.0 mmol/L in the presence of established cardiovascular disease) [1]. In order to achieve these rigorous therapeutic goals, guidelines advocate a stepwise, mechanism-oriented treatment approach. Initial interventions focus on LDL receptor–dependent agents, such as high-intensity statins, ezetimibe, and PCSK9 inhibitors. In cases where the response is deemed to be suboptimal, it is advised that escalation to receptor-independent therapies is considered. Among these, lomitapide, a microsomal triglyceride transfer protein (MTP) inhibitor, occupies a pivotal position due to its unique mechanism of action and demonstrated efficacy in this patient population [1, 6]. Lomitapide is a small molecule inhibitor of the microsomal triglyceride transfer protein (MTP) that effectively reduces the production of apoB-containing lipoproteins in the liver and intestine. Its mechanism of action bypasses LDL receptor pathways, making it particularly suitable for HoFH patients with minimal or absent receptor

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function. Lomitapide has received approval from both the FDA and EMA for use in HoFH, and has demonstrated substantial LDL-C reductions of approximately 50% in both pivotal phase 3 trials and real-world clinical settings [7-1 2]. While the lipid-lowering efficacy and safety of lomitapide are well established [1 3], robust data on its impact on major adverse cardiovascular events (MACE) are still lacking [9, 1 4]. Given the rarity of HoFH (estimated prevalence 1:300,000–500,000) and the ethical constraints of withholding effective therapy, randomized controlled trials (RCTs) are unfeasible in this setting. In order to address this critical evidence gap, the LILITH study was designed as a multicentre, observational, retrospective–prospective cohort study. The aim of the study is to evaluate the incidence of MACE in HoFH patients receiving lomitapide in their routine clinical settings.

Methods

Study design and rationale

LILITH is an observational, multicenter, open-label study with both retrospective and prospective data collection phases. The core design involves comparing the incidence of MACE within the same cohort of patients during two distinct 3-year periods: the three years immediately preceding the initiation of lomitapide treatment (pre-treatment period) and the first three years of lomitapide treatment (treatment period). This intra-patient comparison design was chosen to minimize confounding by indication and by stable patient characteristics, which is crucial in observational studies, especially when a concurrent control group is unavailable, as is typical in rare diseases like HoFH. The study involves multiple lipid centers across Europe and potentially other regions, coordinated by Fondazione SISA (Sponsor). The assignment of patients to lomitapide treatment is determined by routine clinical practice and is independent of study participation. No additional diagnostic or monitoring procedures beyond standard care and lomitapide prescribing information are mandated by the protocol.

Study population

Eligible participants are adult patients (≥18 years) with a clinical or genetic diagnosis of HoFH who have been treated with lomitapide

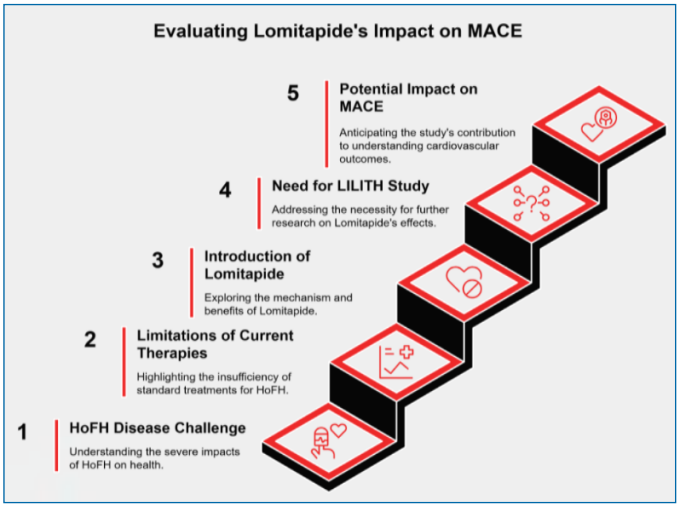

Figure 1 | Schematic representation of the rationale and context of the LILITH study: from the clinical challenge posed by HoFH and the limitations of current therapies, through the introduction of lomitapide, to the need to evaluate the impact of treatment on MACE.

(at any dosage according to prescribing information) for at least 12 months at the time of enrollment. Crucially, patients must have available medical records covering the three years prior to starting lomitapide to allow for retrospective MACE assessment. Patients receiving lomitapide as part of a clinical trial or receiving other investigational agents (other than lomitapide) are excluded. Written informed consent is mandatory prior to any data collection for the study. The target sample size is approximately 72 patients, based on power calculations using preliminary MACE data from European HoFH cohorts [8, 9] (see Section 2.6).

Study periods and data collection

The study encompasses three main periods for data collection:

- **Pre-Treatment period (retrospective):** Data covering the 3 years prior to the first lomitapide prescription (baseline visit) are collected retrospectively from patient medical records. Key data include demographics, medical history (including HoFH diagnosis confirmation), MACE, lipid profiles (total cholesterol, HDL-C, triglycerides, calculated LDL-C), liver function tests (ALT, AST, GGT), and details of all prior lipid-lowering therapies (including LA). Data are collected for baseline and at least one time-point in each of the three preceding years (Y-3, Y-2, Y-1).
- **Treatment period (retrospective/prospective):** Data are collected for the first three years following lomitapide initiation (Y+1, Y+2, Y+3). Data from the first 12 months are collected retrospectively at enrollment. Subsequent data up to Year 3 may be collected retrospectively or prospectively, depending on the patient's enrollment date relative to their lomitapide start date. For patients who have already completed 3 years of treatment at enrollment, data collection can be extended up to 5 years (Y+4, Y+5). Data collected include MACE events, Adverse Events (AEs), Serious Adverse Events (SAEs), Adverse Drug Reactions (ADRs), lipid profiles, LFTs, concomitant medications (especially lipid-lowering therapies), vital signs, weight/BMI. Specific safety monitoring follows lomitapide prescribing information. Exploratory data include liver imaging (ultrasound/MRI for steatosis, elastography/Fibroscan for stiffness, where available per clinical practice), additional biomarkers (ApoB, Lp(a), hsCRP, etc. at baseline and Y+3/Y+5 if available), dietary habits, and medication adherence questionnaires.
- **Post-Study Follow-up:** For patients contributing prospective data, a phone call is made 30 days after their final visit (end of Y+3 or Y+5) to capture any ongoing SAEs or new ADRs/special situations.

Data are entered by site personnel into a secure, web-based electronic Case Report Form (eCRF) compliant with regulatory standards. Source data verification and monitoring are performed by a designated Contract Research Organization (CRO). Medical terms are coded using MedDRA and WHO-ATC dictionaries.

Study Endpoints

- **Primary Endpoint:** The primary outcome is the incidence of MACE during the first three years of lomitapide treatment compared to the incidence during the three years prior to initiation. MACE is defined as a composite of hospitalization for stable or unstable angina, acute myocardial infarction, coronary or carotid revascularization, aortic valve replacement, nonfatal ischemic stroke, transient ischemic attack (TIA), and cardiovascular death. Both first and recurrent events within each period are captured.
- **Secondary Endpoints:**
  - Incidence of 3-point MACE (CV death, nonfatal MI, nonfatal ischemic stroke).

Table 1 | LILITH study protocol procedures and data collection timeline relative to lomitapide initiation.

Protocol Procedures	Pre-Lomitapide			Baseline	On-Lomitapide					
Study time	**Year -3**	**Year -2**	**Year -1**	**First prescription**	**Year 1**	**Year 2**	**Year 3**	**Year 4**	**Year 5**	**Post Study Follow up Day 30 +/- 7 Phone call**
Informed Consent						X	X	X	X	
Inclusion/Exclusion Criteria						X	X	X	X	
Genotype	X									
Demographic Data (sex, age, ethnicity, height)	X									
Demographic Data (weight, BMI)	X	X	X	X	X	X	X	X	X	
Physical Examination and vital signs	X	X	X	X	X	X	X	X	X	
Medical History	X									
MACE	X	X	X	X	X	X	X	X	X	
Plasma lipids (Total Cholesterol, HDL Cholesterol, Triglycerides, LDL-C)	X	X	X	X	X	X	X	X	X	
Liver function test (ALT, AST, GGT)	X	X	X	X	X	X	X	X	X	
Laboratory†				X		X		X		
SAEs	X	X	X	X						
Adverse Events, ADRs, SAEs and Special Situations					X	X	X	X	X	X
Prior and Concomitant Medications	X			X	X	X	X	X	X	
Prior and Concomitant Lipid Lowering Therapies	X	X	X	X	X	X	X	X	X	
Liver MRI or ultrasound				X			X		X	
Liver Elastography				X			X		X	
Medication Adherence Scale						X	X	X	X	
Food frequency questionnaire						X	X	X	X	

  - Incidence of 4-point MACE (3-point MACE + coronary revascularization).
  - Changes in plasma lipids (LDL-C, TC, HDL-C, TG) and LFTs (ALT, AST, GGT) at Y+1, Y+2, Y+3 versus baseline.
  - Changes in concomitant lipid-lowering therapies, including discontinuation or frequency reduction of LA and use of other agents like PCSK9 inhibitors or evinacumab, at Y+1, Y+2, Y+3 versus baseline.
  - Lipid and LFT values during the pre-treatment period, particularly at times when new therapies were initiated.
- **Exploratory Endpoints:** These include changes in additional biomarkers (ApoB, Lp(a), hsCRP, FIB-4, CK-18F etc.), liver steatosis/stiffness assessed by imaging, dietary patterns, and medication adherence, where available. For patients with extended follow-up (Y+4, Y+5), descriptive analysis of MACE, clinical, biochemical, and safety data will be performed.

MACE Adjudication

Given the critical nature of the primary endpoint (MACE) and the potential subjectivity in classifying cardiovascular events based only on local documentation, the protocol mandates a centralized and independent adjudication process. All potential MACE identified by site personnel in the medical records, both in the pre-treatment and treatment periods (3 or 5 years), are reported centrally. An independent Clinical Events Committee (CEC), composed of three expert cardiologists external to the study and without conflicts of interest, will blindly review all relevant source documentation for each potential event (e.g., discharge summaries, ECG reports, imaging results, cardiac enzymes, etc.). The CEC will rigorously apply the predefined MACE endpoint definitions established in the protocol and decide by consensus whether the event meets these criteria. This standardized process is crucial to ensure the reliability, consistency, and objectivity of the primary outcome assessment across different centers and over time.

Statistical Analysis Plan

The primary statistical analysis will compare the proportion of subjects experiencing at least one MACE event during the 3-year treatment period with the proportion of the same subjects experiencing at least one MACE during the 3-year pre-treatment period. McNemar's test for paired nominal data will be used for this comparison, which is the appropriate statistical test for evaluating changes in a dichotomous variable (presence/absence of MACE) measured twice in the same subject (before and after the intervention). The analysis will be two-sided, with a statistical significance level (alpha) set at 0.05. All patients for whom valid MACE data are available for both observation periods will be included in this primary analysis. Secondary continuous endpoints (such as lipid levels and liver enzymes) will

be summarized using standard descriptive statistics (mean, standard deviation, median, interquartile range). Changes from baseline (time of first lomitapide prescription) to the various follow-up time points (Y+1, Y+2, Y+3, and, if applicable, Y+5) will be analyzed using statistical tests for paired data, such as the paired t-test (if data follow a normal distribution) or the Wilcoxon signed-rank test (for non-normally distributed data). Changes in secondary categorical endpoints (e.g., discontinuation of LDL apheresis) will be described using absolute frequencies and percentages, and comparisons between pre- and post-treatment periods may utilize McNemar's test or similar methods for paired categorical data. Safety analyses will include descriptive summaries of the frequency and type of AEs, SAEs, ADRs, and changes in safety-related laboratory parameters (especially ALT, AST, GGT). Exploratory endpoints will be analyzed primarily descriptively. The handling of missing data will be detailed in the Statistical Analysis Plan (SAP). The planned sample size of 72 patients was calculated to provide approximately 80% power to detect a difference in MACE incidence between the two observation periods, assuming event rates similar to those observed in preliminary analyses of European cohorts (e.g., a reduction from ~23% in the pre-lomitapide period to ~11.5% in the post-lomitapide period), with a concordance probability (absence of events in both periods or presence in both) of 88%, using a two-sided McNemar's test at an alpha level of 0.05 [9]. A detailed Statistical Analysis Plan (SAP), specifying all planned analyses, analysis populations, and statistical methodologies, will be finalized before database lock.

Ethical Considerations

The LILITH study protocol has been written in full compliance with the ethical principles enshrined in the Declaration of Helsinki and with the international guidelines for Good Clinical Practice (ICH GCP). Before patient enrollment or any study-specific data collection can begin at a given site, the protocol, the patient informed consent form, and all other participant-facing materials must receive formal approval from the competent independent Ethics Committee (EC) or Institutional Review Board (IRB) for that site, and/or the national regulatory authority, according to local regulations. All participants must provide written, free, and voluntary informed consent after receiving comprehensive explanations about the study. Consent must be obtained before performing any procedure or collecting any information specifically required by the protocol. The confidentiality of patients' personal data is ensured through the use of a unique anonymized subject identification code and secure data management and storage practices, in compliance with data protection regulations as the GDPR in the European Union.

Discussion

The LILITH study employs a specific methodological approach—an observational, multicenter design with retrospective and prospective components and an intra-patient comparison—to evaluate the long-term impact of lomitapide treatment on MACE incidence in the rare and complex HoFH patient population. This design was chosen as a pragmatic and feasible alternative to an RCT, considered unfeasible in this setting for the reasons previously outlined (disease rarity, ethics). The main strength of the intra-patient comparison lies in its inherent ability to effectively control for stable individual-level confounders, i.e., patient characteristics that do not change or change very slowly over time, such as genetic background, disease severity at diagnosis, sex, and chronic comorbidities established before the start of the pre-treatment observation period. This type of control is particularly valuable in observational studies. However, this design is not without potential limitations. A significant limitation is the risk of time-dependent confounding: changes in other therapies (e.g., introduction of new lipid-lowering drugs other than lomitapide), lifestyle, or the status of other comorbidities that may occur differentially between the pre-treatment and treatment periods, and which could influence MACE risk independently of lomitapide. Another potential limitation is the risk of bias in retrospective data collection, such as missing data or incomplete documentation of events, although the use of relatively objective endpoints like MACE (especially if hospitalized) and, crucially, the centralized and independent adjudication process, were implemented precisely to mitigate this risk and standardize outcome assessment. Selection bias is also possible if patients who survive and remain on lomitapide treatment for 3 years systematically differ from those who initiate it. The combination of retrospective and prospective data collection offers a balance between the ability to assess outcomes relatively quickly (by leveraging historical data) and the opportunity to collect more detailed and potentially higher-quality prospective data on aspects like treatment adherence, adverse events, and dietary habits, which may be difficult to reconstruct accurately retrospectively.

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References

[1] Cuchel M, Raal FJ, Hegele RA, et al. 2023 Update on European Atherosclerosis Society Consensus Statement on Homozygous Familial Hypercholesterolaemia: new treatments and clinical guidance. Eur Heart J. 2023; 44(25):2277-2291.

[2] Tromp TR, Hartgers ML, Hovingh GK, et al. Worldwide experience of homozygous familial hypercholesterolaemia: retrospective cohort study. Lancet. 2022; 399(10326):719-728.

[3] Mulder JWCM, Tromp TR, Al-Khnifsawi M, et al. Sex Differences in Diagnosis, Treatment, and Cardiovascular Outcomes in Homozygous Familial Hypercholesterolemia. JAMA Cardiol. 2024; (4):313-322.

[4] Mulder JWCM, Reijman MD, Kusters DM, et al. Homozygous Familial Hypercholesterolemia Is a Life-Limiting Condition: Medical Life-Trajectories in the Post-2010 Era. J Am Coll Cardiol. 2025; 85(19):1898-1903.

[5] Mulder JWCM, Kranenburg LW, Treling WJ, et al. Quality of life and coping in Dutch homozygous familial hypercholesterolemia patients: A qualitative study. Atherosclerosis. 2022; 348:75-81.

[6] Arca M, D'Erasmo L, Cuchel M, et al. Long-term experience with lomitapide treatment in patients with homozygous familial hypercholesterolemia: Over 10 years of efficacy and safety data. J Clin Lipidol. 2025; 19(4):775-789.

[7] Cuchel M, Meagher EA, du Toit Theron H, et al. Efficacy and safety of a microsomal triglyceride transfer protein inhibitor in patients with homozygous familial hypercholesterolaemia: a single-arm, open-label, phase 3 study. Lancet. 2013; 381(9860): 40-46.

[8] Underberg JA, Cannon CP, Larrey D, Makris L, Blom D, Phillips H. Long-term safety and efficacy of lomitapide in patients with homozygous familial hypercholesterolemia: Five-year data from the Lomitapide Observational Worldwide Evaluation Registry (LOWER). J Clin Lipidol. 2020; 14(6):807-817.

[9] D'Erasmo L, Steward K, Cefalù AB, et al. Efficacy and safety of lomitapide in homozygous familial hypercholesterolaemia: the pan-European retrospective observational study [published correction appears in Eur J Prev Cardiol. 2022; 29(13):1812]. Eur J Prev Cardiol. 2022; 29(5):832-841.

[10] D'Erasmo L, Giammanco A, Suppressa P, et al. Efficacy of Long-Term Treatment of Autosomal Recessive Hypercholesterolemia With Lomitapide: A Subanalysis of the Pan-European Lomitapide Study. Front Genet. 2022; 13:937750. Published 2022 Aug 22.

[11] Pavanello C, Suppressa P, Castiglione S, et al. Sex-related differences in response to lomitapide in HoFH: A subanalysis of the Pan-European Lomitapide retrospective observational study. Atherosclerosis. 2025; 401:119089.

[12] D'Erasmo L, Gallo A, Cefalù AB, et al. Long-term efficacy of lipoprotein apheresis and lomitapide in the treatment of homozygous familial hypercholesterolemia (HoFH): a cross-national retrospective survey. Orphanet J Rare Dis. 2021; 16(1):381. Published 2021 Sep 8.

[13] Larrey D, D'Erasmo L, O'Brien S, Arca M; Italian Working Group on Lomitapide. Long-term hepatic safety of lomitapide in homozygous familial hypercholesterolaemia. Liver Int. 2023; 43(2):413-423.

[14] Suppressa P, Coppola C, Cocco V, O'Brien S. Long-term effectiveness and safety of lomitapide in patients with homozygous familial hypercholesterolemia: an observational case series. Orphanet J Rare Dis. 2024; 19(1):370. Published 2024 Oct 8.