Scientific Research Project Number: MA 2016 010
Place: AMC, dept. of Gastroenterology and Hepatology

Place of the SRP Project

Department of Gastroenterology and Hepatology
Academic Medical Center
Clinical Research Unit
Academic Medical Center

Introduction

Primary sclerosing cholangitis (PSC) is an uncommon cholestatic liver disease. Obstruction of bile in these patients results in recurrent cholangitis, pruritus, fatigue an upper abdominal pain. In the more advanced disease stage patients are at risk of developing liver cirrhosis and hepatobiliary and colorectal malignancies. Liver transplantation is the only curative treatment option. Therapy mainly focuses on postponing liver transplantation and improving quality of life. Ursodeoxycholic acid (UDCA), a drug that influences bile composition, is the only available medical treatment. However, it has never been proven to be (cost-) effective due to the rarity of PSC and the high costs for a randomized controlled trial. Moreover, confounding and the heterogeneity of the patient population complicates such research. Most patients have co-existing inflammatory bowel disease and use a lot of other drugs. Also, there is no distinct classification for the different disease stages.

Our hypothesis is that UDCA is cost-effective in PSC patients. We have a retrospective cohort with approximately 600 PSC patients. Most of them will be followed prospectively. All patients will receive periodic questionnaires with questions on their health status, for example medication use, medical consumption and disease progression. With this information we will be able to do a cost-effectiveness analysis.

Description of the SRP Project

Problem

To perform a cost-effectiveness analysis, two different modeling strategies can be used, patient-level modeling and cohort-level modelling. Until now, we have not been able to determine the optimal approach for this project. In order to make a careful decision on which model to use, we want to explore both of them. The exploration of these two strategies will be the main focus of this SRP.

Research Questions

1. Which modelling strategy suits best for cost-effectiveness analysis in PSC patients, patient-level modelling or cohort-level modelling?

Results

1. An exploration of the different disease models (built in Microsoft Excel, DataTriage or another program)
2. An advice on which modeling strategy to use for the cost-effectiveness analysis.
3. Results from a pilot cost-effectiveness analysis in the different models.
4. A protocol for data handling (input in database, data retrieval and input in SPSS) in order to be able to perform the cost-effectiveness analysis.

Time period

8 months

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