# Common Drug Review
## *Patient Group Input Submissions*

**Repatha (evolocumab)** for primary hyperlipidemia and mixed dyslipidemia

| **Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.** |
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| FH Canada Patient Network — permission granted to post. |
| Heart and Stroke Foundation — permission granted to post. |

**CADTH received patient group input for this review on or before Date**
The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations.

While CADTH formats the patient input submissions for posting, it does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

# FH Canada Patient Network

## Section 1 — General Information

| | | |
|---|---|---|
| **Name of the drug CADTH is reviewing and indication(s) of interest** | | Repatha (evolocumab) |
| **Name of the patient group** | | FH Canada Patient Network |
| **Name of the primary contact for this submission:** | | [redacted] |
| Position or title with patient group | | [redacted] |
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| | Address | 151 Bloor Street West, Suite 600 |
| | Website | http://www.fhpatientcanada.org |
| **Permission is granted to post this submission** | | Yes |

### 1.1 Submitting Organization

The FH Canada Patient Network is a volunteer-led national non-profit organization. The FH Patient Network was organized with the direct assistance of clinicians in Montreal and Vancouver, with outreach through the FH Canada Registry Network and the FH Foundation in the USA. The purpose of the FH Canada Patient Network to raise awareness about Familial Hypercholesterolemia (heterozygous and homozygous), to promote screening and diagnosis, to provide education about the condition, to improve access to appropriate treatment and care, and to provide a forum for advocacy and support. The FH Canada Patient Network receives organizational and administrative support from the Consumer Advocare Network, a not-for-profit network that promotes the capacity of patient organizations to participate in healthcare policy and decision-making. The FH Patient Network participates in the establishment of FH International and FH Europe.

### 1.2 Conflict of Interest Declarations

The FH Canada Patient Network receives unrestricted educational grants from Sanofi Canada, Pfizer Canada, Amgen, and Aegerion. It receives organizational and administrative support from the not-for-profit Consumer Advocare Network at no cost. There are no other conflicts of interest.

## Section 2 — Condition and Current Therapy Information

### 2.1 Information Gathering

The FHCPN is collecting information in support of FH patients but also on behalf of all patients with high cholesterol who could be appropriate candidates for Repatha based on the clinical trials and anticipated regulatory approval by Health Canada. Moreover, because many patients with FH have not actually been diagnosed (international statistics suggest only about 20% are diagnosed), we felt it was important to reach out to the broader patient population. Our outreach extended to all patients with familial and nonfamilial hypercholesterolemia, that is, low-density lipoprotein cholesterol (LDL-C) that is not controlled (not at target levels).

Because there are no patient organizations in Canada with direct access to this broad category of patients, we chose to use a variety of sources to recruit participants, namely:

- Patients, families and providers who had made direct contact with FHCHN
- Institutional sites in Canada conducting clinical trials with Repatha (six sites)
- Social media including sites for FH Canada Patient Network, FH Patient Canada Facebook, FH Foundation, The Familial Hypercholesterolemia (FH) Foundation Patient Discussion Group, Familial Hypercholesterolaemia (FH) Discussion Group, PCSK9 Forum, and Twitter.

The following methods were used to collect information: One-on-one interviews; focus groups, survey posted on Survey Monkey; questions posed in online forums and closed discussion groups.

We received input from a total of 105 respondents including patients and caregivers (some patients were also caregivers). Respondents varied in terms of their genetic risk factors, with about one-fourth reporting no history or symptoms of FH, about one-third diagnosed with FH (mostly HeFH but also four with HoFH), about one-third reporting a family history of FH (probably with FH themselves) and the remainder (about 15%) said they were not sure of any FH. About three-fourths identified themselves as residing in Canada, about half in Quebec and the remainder mostly in Ontario with a few in Manitoba and Alberta. The remainder identified either as residing in the USA or "unspecified."

**Patient characteristics.** All of the patients self-identified as currently or in the past having "high cholesterol" that was or is "not controlled", that is, higher that target levels. About two-fifths have experience with Repatha, almost evenly split between familial and nonfamilial. Among those who have not used Repatha, about one-fourth said their current therapy was working well or moderately well to manage their cholesterol. Conversely, about one-fourth said their therapy was "not at all" working, with the remainder saying it was mostly not working. Among those on Repatha, all reported their cholesterol had been lowered near or at target. Because the numbers representing each patient type are small and because their responses do not differ based on their FH status, the results across familial and nonfamilial are combined, except where meaningful differences are relevant. Clearly, the challenge of managing "uncontrolled" high cholesterol is a common and overwhelming influence in their responses. In terms of other risk factors, about 20% said they smoked (now or in the recent past); about one-half identified "excessive weight" as a factor, with similar numbers for diet and exercise. More than half said they have or are at risk for diabetes.

### 2.2 Impact of Condition on Patients

**What aspects of this condition are more important to control than others?** All participants identified "getting or keeping" their cholesterol low as their primary concern. Most, currently or in the past, experienced some challenges in achieving their goal. There were two main types of challenges. For some (including those pre-Repatha), their cholesterol levels were not sufficiently lowered despite their

current (or previous) treatments, including drugs, diet, exercise, natural foods, and other therapies. Others reported side effects that made their treatment difficult to maintain or tolerate, namely, muscle pain (especially in legs and ankles), chest pains, headaches, fatigue and weakness, anxiety and depression. Most have tried several treatment regimens, including different statins and statin combinations, diet, natural therapies, and other drugs.

**How does this condition affect day-to-day life?** About three-fourths (pre-Repatha and not on Repatha) said they had not been able to sufficiently lower their cholesterol despite their prescribed treatments. However, we do not know the degree to which these respondents are typical of all patients with high cholesterol nor do we know the percentage of patients with high cholesterol who are seeking alternative therapies. Many persons living with high cholesterol are inured to their status.

Most of the FH patients and more than half of the nonFH patients reported having had at least one cardiovascular intervention, that is, angioplasty, bypass and/or stent. Many have had multiple events requiring multiple interventions. About one-third of patients with FH and all of those with HoFH said they had symptoms either regular or occasionally. These include shortness of breath, fatigue, muscle, joint and chest pain, and headaches. *"I am on apheresis at least every two weeks, and the last time, I started to get severe chest pains right there in the clinic. I was rushed to emergency where they decided to put in another stent."*

Importantly, about three-fourths of the nonFH participants and about half of those with FH reported that the day-to-day symptoms of high cholesterol were experienced as minor or nonexistent. Indeed, when they were not symptomatic, most did not think of high cholesterol as a chronic condition or consider themselves to be patients. However, those who have a history of cardiovascular disease in the family or have personally experienced cardiovascular events were more likely to think about the risks. Nevertheless, all said that even when they were not suffering from symptoms directly related high LDL, they lived with the stress and fear of a potential or next cardiovascular event, which can be debilitating and even deadly. Overall, the challenge of achieving or maintaining low cholesterol is a constant concern that affects their lives on a daily basis in many ways. We summarize their responses as follows.

First, patients reported stress, anxiety, and frustration with not being able to get or keep their cholesterol to target levels. The impact was as much emotional as it was physical. They reported being "accused" by their health professionals of not adhering to their medications. *"I take them religiously but the doctor just doesn't want to believe me, and keeps increasing the dosage or changing the meds. Now I feel like I've run out of options."* They also reported being made to feel guilty about their weight or lack of exercise. *"No matter how hard I try, nothing seems to work. I just feel like giving up."*

A second category of expressed concerns focused on the implications of the "high numbers" for their health, especially the risk of stroke or other cardiovascular event. Some have had cardiovascular surgery (angioplasty, bypass, and/or stents), For those patients with FH, the fear of death is very real, since many reported that they had a parent, grandparent, and/or other relatives who have died of heart attacks, often at a very early age (pre-40's or 50's). *"My father died suddenly of a heart attack at age 39 and my aunt (his sister) died at age 50. I know I have FH and I need to do everything I can to keep my [cholesterol] numbers down." "I've been on three different statins and now on a statin with another drug but I can't seem to get my cholesterol level below 15."* "*My doctor says I just need to lose weight and eat better but I don't think that will help. My numbers were high when my weight was much lower."*

For many patients, a third type of on-going challenge is not the condition but the side effects of the (statin) therapy. *"I had switched statins and found myself spiralling into the worst depression. My doctor didn't believe it was related but switched me back and things are back to normal. I'd rather struggle with the cholesterol than experience that again." "I thought I was having a heart attack but it turns out to be panic attacks; I switched to ezetimibe and that worked for a while." "I've tried every*

*statin, high dose and low dose, but the pains in my legs, especially my calves, were so bad that I couldn't walk even as far as the bus stop. What else can I do?" "I switched from atorvastatin to rosuvastatin because I was having extreme pains in my legs and hips. The pain has gone, but now I'm just exhausted all the time. Someone suggested taking CoQ10 and vitamin D but that brings me up to 11 medications."*

For patients on apheresis, the time required is perceived as highly disruptive. In some cases, patients also reported having to travel considerable distances (3 to 4 hours) to get to the appropriate apheresis site several times a month. For many, the improvement in their symptoms (shortness of breath, chest pains, anxiety and vision impairment) did not last very long and *"the period of time between feeling normal and feeling lousy seems to get shorter and shorter."* *"I am on apheresis at least every week, which is interferes with my work." "The last time I was there, I started to experience severe chest pains and was rushed to emergency to have another stent put in."*

Overall, among patients not on Repatha, about one-fourth say their current therapies are working to lower cholesterol but three-fourths reported their cholesterol is not being managed sufficiently (with and without additional symptoms). Most express frustration with trying different regimens with limited success and are fearful of potentially serious CV events. Finally, some are experiencing significant side effects (muscle pains) with their current therapies (mostly statins) and are seeking more tolerable alternatives. FH and nonFH patients express similar challenges and concerns

### 2.3 Patients' Experiences With Current Therapy

This question was partly answered in the previous section. All patients have been on statins, with about one-fourth no longer taking statins and about half of those taking no medications (at this time). About one-fourth (not on Repatha) reported that their current therapies are working effectively to lower their cholesterol, if not to target at least to a level where they (and their physician) are not worried. Among these, most are on statins or a combination of statin and ezetimibe. About 10% are also on apheresis (FH), though the reported effects are mixed. One person said they experienced a significant improvement in LDL levels; however, she had only been on the therapy for a short period of time and was not sure of the long-term benefit. Another patient (with HoFH) has been on apheresis therapy for about four years, and reported, *"It (apheresis) is definitely no longer working. I have had with multiple hospitalizations and stents inserted on an emergency basis, but they don't working for long."* Other patients expressed concerns about the time needed for apheresis. *"It means I can no longer work fulltime, which means that my wife has gone back to work, even though we have two small children."*

The reported side effects to drugs other than Repatha varied among patients. Almost all of the patients reported having experienced some side effects that were likely related to statins or a statin combination, either currently or in the past. Some said these lessened over time as they adjusted to the medicines. About three-fourths of the patients said they were not managed sufficiently on the statins or had serious adverse reactions with their other therapies. Overall, the most frequent and difficult side effect to statins was muscle pains (myopathy), which were sometimes significant enough to require lowering the dosage, taking a break from treatment, discontinuing the statin altogether, switching statins, or switching to another therapy with or without a low-dosage statin. Some patients reported that the "drug holiday" or the switch was effective; others continued to have side effects (with the same or another statin) or experienced insufficient response to the alternative therapy.

Other side effects attributed to statins were anxiety and depression, fatigue, and chest pains. However, as several respondents pointed out, it is hard to know whether the symptoms were due to their primary condition [of high cholesterol], to the drugs, or to wrong dosage. Several patients discussed the challenges of switching therapies. Some couldn't convince their doctors to let them try something else (at least initially) and others said their meds were switched without their knowledge. *"I told the doctor I was experiencing some side effects (dizziness, bladder control, and anxiety) from my new meds, and he*

*said that was not possible. He said he had just moved me from the [brand] statin to the same generic version. I convinced him to switch me back, and the side effects went away."*

The challenges with apheresis have already been presented. For those who had been on apheresis for a number of years, there seemed to be initial relief from the symptoms related to FH but over time, the benefits lessened and the adverse effects and challenges seemed to increase. Some patients also reported that adhering to a strict diet was very difficult with FH and that healthcare professionals were "clueless" and unsympathetic about the challenges. Moreover, patients reported that the dietary restrictions varied depending on which healthcare professional they talked to. *"The Mediterranean diet sounds fine but it is not easy to do all the time; it can also get costly and tedious."*

In summary, we do not know how many Canadian patients with hypercholesterolemia (familial or not) are unresponsive to, intolerant of, or contra-indicated for statins. We do know that many patients simply reduce or stop taking statins when the side effects become intolerable. Some will ask for an alternative but others will not do anything until they experience a cardiovascular event.

### 2.4 Impact on Caregivers

Many of the caregivers we heard from were parents of children diagnosed with or at risk for FH. Some of the children were as young as 8 or 9 years of age. Most said they had suspected FH because of the family history and some indicated it was a relief to have a diagnosis. While obviously anxious about the condition, most were resolved to start therapy when necessary and were optimistic about the long-term outcomes. Some caregivers expressed frustration with getting their spouses or older children to stay on therapy, especially when they seemingly experienced no immediate benefit or negative outcome when not followed. Caregivers supporting or managing patients with multiple conditions, including Lupus, MS, Parkinson's, and diabetes, found the many medications and the dietary recommendations especially difficult. *"The medication schedule requires that you not take one pill while doing another; you have to space them out and it is not so easy when the [person] is not there with you 24/7." "We've had to look up all the medications ourselves since the various doctors don't seem to have clue as to what others have prescribed. If it weren't for my pharmacist, I think we would have been in serious trouble."*

## Section 3 — Information about the Drug Being Reviewed

### 3.1 Information Gathering

Same sources and methods as described as in Section 2.1

### 3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

For those with no experience using evolocumab (or any PCSK9), about three-fourths were aware of the new therapy. Among FH patients, almost four-fifths were anticipating approval. There was not much difference in the opinions of those who were well managed and those who were not. Regardless of whether the participant was sufficiently managed on statin (or other therapy), he/she expected that evolocumab would be a valuable alternative to statins for lowering cholesterol levels. For some, this was based on their belief (and previous experience) that all "cholesterol lowering" therapies were effective only for a period of time, whether it was because their bodies had adapted to the therapy, they developed resistance to the specific therapy, or their condition had changed necessitating a different therapy. *"My first statin was fine but I had to increase the dose as my system became adapted to the drug and then I had to add another drug (ezetimibe) to boost the effectiveness of the statin. I expect I'll be prescribed Repatha when I need to switch up again." "Over time, I seemed to be getting more side effects that were not going away and my cholesterol was not going down the way it used to. I had read*

*that Repatha works differently from the statins so I asked the doctor about whether it might work for me. He said it might be the next option." "When I was diagnosed with Lupus, I had to start two other medications, so my doctor took me off the statins temporarily. When I started back, they just didn't seem to have the same effect and my cholesterol was stuck well over 30. I like the fact that you don't have to take [Repatha] every day so it might not be a problem with my other medications."*

For the participants not currently at target levels, most expected that Repatha would work to lower cholesterol levels, possibly more effectively and without the side effects experienced with statins. For patients with HeFH and HoFH not managed on current therapies, the expectation was that Repatha would work even though other therapies had failed. Moreover, some said that based on how the PCSK9s worked, they expected their cholesterol to remain at normal levels. For parents, Repatha (PCSK9) offered the first "real hope" that their children could avoid strokes or heart attacks. For all patients, there was a strong sense that Repatha (PCSK9) could be the "final" therapy, allowing them to stop the constant checking and changing of medicines. Many indicated that they would be able to eat "more normally" and not be so concerned about adhering strictly to low fat or vegetarian only diets.

Moreover, all participants indicated that they were not concerned or only somewhat concerned about administering an injection. Similarly, they did not feel it would be difficult to remember to take the therapy either once or twice a month. However, it is important to note that these participants also said that taking a daily pill was also not difficult (so these are self-reported highly compliant patients).

For patients who have received Repatha through the clinical trials, all were satisfied or very satisfied with the impact on their cholesterol level. They spoke of both physical and emotional (psychological) benefits. *"I noticed a difference almost right away and my numbers stayed down, even between injections." "This is the first time in a long time that I felt some hope in getting on top of this condition. I had almost given up on getting my cholesterol under control." "I feel fantastic!" "I feel so lucky that my doctor enrolled me in the clinical trials. I could tell almost right away that I was on the real therapy and I was right. Like two weeks, and the difference was huge."* Among the patients who have been on evolocumab, all said they were still on therapy, although some were also taking statins. All said their cholesterol levels have remained close to target or lower than before taking evolocumab. *"With my previous drugs [statins], my doctor was constantly checking my cholesterol and making changes to my medicines. This has been working for me without almost no problems." "I find it so much easier to do the injections once a month than taking the pills every day. And with the new pen, it is even easier."*

Most of the patients experienced few or no lasting adverse reactions to evolocumab and commented on how they appreciated being free of the side effects they had experienced with statins. *"I had some soreness with the injections initially but that has gone away almost completely." "It was such a relief to be able to walk without pain; I've even lost weight now that I am back to regular exercise." "I thought it would be difficult to give myself an injection but I got the hang of it pretty quickly."* None of the patients in the survey had discontinued evolocumab, although some were worried about the eventual cost of the therapy and whether it would be covered. *"I can't imagine going back to statins or something else. Maybe, now that my cholesterol is down, they would work but I don't want to take the chance."*

In summary, all participants were overwhelmingly positive about Repatha as an important alternative for managing high cholesterol. While we would not suggest that these responses are representative of the entire hypercholesterolemia population, there is no doubt that evolocumab is a treatment of choice for patients who have had challenges in lowering their cholesterol levels with other therapies, who have had serious adverse reactions to statins or statin combinations, or who have a history of FH. When asked directly whether they would choose a daily pill or an injection if both worked equally, most said they had "no preference." However, when asked if they would choose a monthly injection over a pill if the injection "worked better" or if there were fewer serious side effects, all chose the injection.

## Section 4 — Additional Information

In cases like this, more time is needed. It was very difficult to identify and recruit participants appropriate for this drug. Most FH patients are undiagnosed, and most people with high cholesterol are not members of patient groups. Many clinicians do not understand the Common Drug Review process and do not appreciate the value of the patient submission. They are reluctant to take the time to identify and refer clinical trial participants. I was fortunate in that my husband has FH and as a founder of the FHCPN, I had good relationships with several of the clinicians. And because I have done submissions for other patient groups, I was able to use a variety of strategies including outreach through the social media and through other networks. However, it would be much more effective to have a database of patients recruited at the time of the clinical trials who would be willing to provide input at the time of submission. Also, there should be more flexibility on space, since this is a complex and varied population with different needs and potential benefits/risks that could have been better presented to allow for differential access, if necessary.

# Heart and Stroke Foundation

## Section 1 — General Information

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| **Name of the drug CADTH is reviewing and indication(s) of interest** | Repatha |
| **Name of the patient group** | Heart and Stroke Foundation |
| **Name of the primary contact for this submission:** | [redacted] |
| Position or title with patient group | [redacted] |
| Email | [redacted] |
| Telephone number(s) | [redacted] |
| **Name of author (if different)** | |
| **Patient group's contact information:** Email | cadth@hsf.ca |
| Telephone | (613) 691-4062 |
| Address | 1402-222 Queen Street, Ottawa, ON |
| Website | www.heartandstroke.ca |
| **Permission is granted to post this submission** | Yes |

### 1.1 Submitting Organization

The Heart and Stroke Foundation of Canada (HSF), a volunteer-based health charity, leads in eliminating heart disease and stroke and reducing their impact. Its mission is to prevent disease, save lives, and promote recovery.

The Heart and Stroke Foundation is one of Canada's largest and most effective health charities. Over the last 60 years we have invested more than $1.39 billion in heart and stroke research, making us the largest contributor in Canada after the federal government. In that time, the death rate from heart disease and stroke has declined by more than 75 per cent.

The Foundation's health promotion and advocacy programs across the country are saving lives every day. Working together, our employees, volunteers, donors and world-class researchers have made the Heart and Stroke Foundation what we are today: Canada's most widely recognized and trusted authority on cardiovascular health. Our vision is healthy lives free of heart disease and stroke. Together, we will make it happen.

The Heart and Stroke Foundation is a national organization led and supported by a force of about 125,000 volunteers.

### 1.2 Conflict of Interest Declarations

The Heart and Stroke Foundation of Canada (HSF) and the individuals involved in the preparation of this submission have no conflict of interests to declare. While the majority of HSF funding comes from individual donors, HSF has received unrestricted financial support from pharmaceutical companies to help us achieve our mission of preventing disease, saving lives and promoting recovery. This financial

support is used for the development of educational materials; education, awareness and community engagement activities; and funding of research awards across the country. Over the past five years, HSF has received unrestricted financial support from: Aegerion Pharmaceuticals, Amgen, Apotex, AstraZeneca, Bayer, Boehringer Ingelheim, Bristol-Myers Squibb Canada, Eli Lily Canada, GlaxoSmithKline Inc., Janssen, McKesson Canada, Merck, Merz Pharma Canada, Novartis, NovoNordisk, Pfizer Canada Inc., Sanofi, Servier, Takeda, and Valeant.

There is no conflict of interest.

## Section 2 — Condition and Current Therapy Information

### 2.1 Information Gathering

The information on condition impact to patients and caregivers was gathered by HSF through an online survey using the 'Survey Monkey' tool. Access and links to the survey were advertised using targeted, promoted posts through Facebook and pop-ups on cholesterol risk factor information pages of the HSF public website (www.heartandstroke.ca). The survey was made available to the public from July 6, 2015 to July 22, 2015.

In total, 32 individuals started the online survey and 28 completed the survey. Participants were not obligated to complete all questions in the survey. Participants were asked whether they have ever been told by a healthcare professional that they have familial hypercholesterolemia. Of the 32 individuals who responded, 23 individuals indicated that they have familial hypercholesterolemia. Participants were also asked whether they are a caregiver for someone who has familial hypercholesterolemia. None of the respondents indicated that they were a caregiver for someone with this condition. Responses from participants that answered yes to a diagnosis of familial hypercholesterolemia (n=23) were used to inform this submission.

Information was also generated through literature searches from peer reviewed publications, Heart and Stroke Foundation health information and guidelines and policies from credible organizations such as the Canadian Cardiovascular Society. The Heart and Stroke Foundation develops guidelines, policies and position statements that are based on scientific evidence. These guidelines, policies and position statements form the basis of the health information that is provided by HSF to the public, health professionals and the media in various formats (print, web, CPR training materials, media releases, etc.).

Limitations: This survey was not a population based survey. Very few responses were obtained through the online survey which provides limited data to inform this submission. This submission reflects the views and/or experiences of a small number of survey respondents and is not representative of the views of all patients with familial hypercholesterolemia or their caregivers living in Canada.

### 2.2 Impact of Condition on Patients

Heterozygous familial hypercholesterolemia affects approximately 1:500 Canadians, and the more serious homozygous form affects approximately 1:1,000,000 Canadians, although these numbers might be underestimated. Approximately 83,500 Canadians are estimated to have familial hypercholesterolemia yet most are undiagnosed.i

Survey participants were asked if they had ever been told by a healthcare professional that they have familial hypercholesterolemia. A total of 23 survey participants identified as having familial

hypercholesterolemia. Responses from these individuals are reported below. Respondents were not required to complete all questions in the survey.

As a result of this condition, 6 patients of a possible 23 said that it has affected their day-to-day life because they have to take medication at specific times. One patient reported that they have to visit a healthcare provider frequently as a result of this condition and another indicated that they have to manage the condition with other forms of therapy. One patient commented "I was told I had a stroke but no proof of a stroke or heart attack was proven, I simply fell because I was tired and overworked."

Seven patients of a possible 23 indicated that having familial hypercholesterolemia has not affected their ability to do activities. One individual reported that familial hypercholesterolemia has affected their day-to-day life but did not provide additional details.

When asked about symptoms related to this condition, two patients reported that they have experienced symptoms as a result of this condition. One patient reported experiencing fatigue, while another noted arteriosclerosis and heart stents.

## 2.3 Patients' Experiences With Current Therapy

Nine patients with familial hypercholesterolemia indicated that they have been prescribed medication to control their blood cholesterol levels. None of the respondents had been prescribed Repatha by their healthcare provider or as part of a clinical trial.

Of the nine patients prescribed medication to control their condition, the most common medications prescribed were statins including atorvastatin/Caudet/Lipitor (n=4), rosuvastatin/Crestor (n=3), pravastatin/Pravachol (n=1), simvastatin/Zocor (n=2), and statins (n=2). Two patients reported being prescribed ezetimibe/Ezetrol and one patient reported taking Ramipril (in addition to atorvastatin) for treatment of high blood cholesterol. Five of the nine respondents are actively taking medication to control this condition (having last taken medication 'today' or 'yesterday'). Four respondents reported taking medication more than one year ago. Seven respondents noted that these medications have helped to control this condition, while one patient reported that taking medication has not helped to control their condition. Three patients need to take more than one medication. There were no concerns regarding access to these medications.

Seven of the nine patients prescribed medication to control or prevent high blood cholesterol experienced unwanted side effects as a result of taking medication. The unwanted side effects included: a feeling of 'pins and needles' (a sensation of burning, pricking, tickling or tingling in the hand, arms, legs, feet or other part of the body) (n=4); muscle weakness, tenderness or spasms (n=4); pain in the joints, back, arms, legs or other parts of the body (n=4); sleep issues (inability to sleep, restlessness, disturbed sleep, wakefulness, insomnia) (n=3); dry mouth or altered taste (n=2); gastrointestinal issues (abdominal pains/cramps, constipation, diarrhea or flatulence) (n=2); headache (n=2); skin reactions (rash, itching) (n=1); dizziness, nausea, vomiting (n=1); fatigue (n=1), tongue swelling (n=1) and elevated blood sugar levels (n=1).

### 2.4 Impact on Caregivers

There were no respondents who identified themselves as a caregiver for someone with familial hypercholesterolemia.

## Section 3 — Information about the Drug Being Reviewed

### 3.1 Information Gathering

Information to complete Section 3 was gathered in the same way as Section 2. Please refer to section 2.1 for further information on this process.

### 3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

None of the respondents were taking Repatha to control their blood cholesterol. Six patients responded to the question 'Other than being cured, what would be the best course of treatment look like for you?" Responses included "something without serious side effects or liver disease", "exercise, eating a healthy diet, rest, balanced lifestyle', 'I'm tired of pills", "continue with medication" and "just to keep my cholesterol at a low normal level".

There were no patients taking Repatha therefore we are unable to complete this section of the submission.