

## CADTH Reimbursement Review

# Patient Input

### ISATUXIMAB FOR INJECTION (Sarclisa)

Sanofi Genzyme, a division of Sanofi-aventis Canada Inc.

**Indication:** Multiple myeloma

**CADTH received patient input from:**

Myeloma Canada

**August 9, 2021**

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CADTH 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.

**Myeloma Canada Patient Input — Sarclisa (isatuximab for injection) with carfilzomib (Kyprolis) and dexamethasone**

<b>Name of the Drug and Indication</b>	Sarclisa (isatuximab for injection) with carfilzomib (Kyprolis) and dexamethasone
<b>Name of the Patient Group</b>	Myeloma Canada
<b>Author of the Submission</b>	[REDACTED]
<b>Name of the Primary Contact for This Submission</b>	[REDACTED]
<b>Email</b>	[REDACTED]
<b>Telephone Number</b>	[REDACTED]

**Appendix A: pCODR Patient Group Conflict of Interest Declaration**

1. *Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.*  
**No**
2. *Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.*  
**No**
3. *List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.*  
**Amgen, Sanofi, Janssen, BMS, Celgene, Takeda, Merck, Pfizer, Karyopharm, Novartis, GSK, Leo and Rapid Novor.**

*I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.*

**Name: Martine Elias**  
**Position: Executive Director**  
**Patient Group: Myeloma Canada**  
**Date: July 17, 2021**

**Appendix B: Patient Input Template for CADTH CDR and pCODR Programs**

1. **About Your Patient Group.**  
See registration information  
[www.myeloma.ca](http://www.myeloma.ca)

## 2. Information Gathering

Over the years, Myeloma Canada has collected data on the impact of myeloma and its treatments on patients and caregivers, by conducting several patient and caregiver surveys. The compiled data are then presented to the Pan-Canadian Oncology Drug Review. Myeloma Canada is sharing patient input from one such survey regarding the combination of Sarclisa (isatuximab for injection) with carfilzomib (Kyprolis) and dexamethasone. Our survey was available from April 22<sup>nd</sup> to May 9<sup>th</sup> 2021, and was shared with patients across Canada, via email and social media. 208 patients answered the survey, from Alberta (26), British Columbia (27), Manitoba (6), New Brunswick (3), Newfoundland and Labrador (3), Nova Scotia (10), Ontario (86), Prince Edward Island (1), Quebec (38), Saskatchewan (5), Yukon (2), and (1) from outside of Canada. No patients from Nunavut or Northwest Territories responded, and a total of 48 did not meet the eligibility criteria thus their responses excluded from the report. Eligibility for the survey was determined by patients having received at least one prior line of therapy; eligible patients were then divided into 3 subsets based on the following criteria.

- Subset 1: patients (15) had received the carfilzomib (Kyprolis) and dexamethasone combination only. Respondents of Subset 1 were from British Columbia (3), Ontario (7), Quebec (5). (Data specifically discussed in section 4)
- Subset 2: all other **eligible** respondents who had no experience with the treatment combination under review, or carfilzomib (Kyprolis) and dexamethasone alone.
- Subset 3: patients (3) who received the treatment under review. Respondents were from Ontario (1), Saskatchewan (1), and Québec (1). The respondent from Ontario did not respond to the majority of questions related to the treatment under review, and the respondent from Quebec was determined, based on their comments, to have confused the treatment combination under review, with another treatment and, did not receive isatuximab (for injection) with carfilzomib (Kyprolis) and dexamethasone, thus their results excluded from section 6<sup>1</sup>. (Data specifically discussed in section 6)

## 3. \*Disease Experience

Every day, 9 Canadians are diagnosed with myeloma. Despite its growing prevalence, the disease remains relatively unknown. To date there is no cure for myeloma. With myeloma, abnormal plasma cells (also known as myeloma cells) interfere with the production of normal healthy blood cells in the bone marrow and overproduce inactive clones of abnormal antibodies that can negatively affect different parts of the body such as the bones and kidneys. The cause(s) of myeloma remain unknown. Myeloma is what is known as a relapsing-remitting cancer; alternating between periods of remission that require no treatment, and symptomatic periods in which complications arise that require treatment, but it will ultimately always return to the latter.

When asked “How important it is for you to control various aspects of myeloma (Please rate on a scale of 1 ‘Not important,’ to 5 ‘Very important’)”, 158 eligible respondents identified the following symptoms as very important: infections (68.59%), kidney problems (56.69%), mobility (48.39%), neuropathy (42.04%), and fatigue (42.04%).

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<sup>1</sup> « Récidive au mois d’octobre 2020. Depuis octobre 2020, début d’un traitement avec Daratumumab, Velcade et dexaméthasone. Je vais très bien !! » The Québec patient data’s removal was based on this comment.

\* This section presents data from all survey respondents: all three patient subsets combined.

In answer to the question, “How important it is to you to have access to effective treatments for myeloma (Please rate on a scale of 1—Not important to 5—Very important)”, 96.25% (154) of respondents (160) selected “5—extremely important.”

When asked to “Rate on a scale of 1–5 (1 is ‘Not at all’, and 5 is ‘Significant impact’), how symptoms associated with myeloma impact or limit your day-to-day activities and quality of life.” 158 patient responses showed myeloma significantly impacted their ability to work (28.66%), to travel (25.95%), to concentrate (19.75%), and to exercise (19.62%).

	1 - NOT AT ALL	2 - HAS A LITTLE IMPACT	3 - HAS AN IMPACT	4 - SOMEWHAT OF AN IMPACT	5 - SIGNIFICANT IMPACT	N/A	TOTAL	WEIGHTED AVERAGE
Ability to work	8.92% 14	17.20% 27	12.74% 20	10.19% 16	28.66% 45	22.29% 35	157	3.42
Ability to travel	7.59% 12	13.92% 22	20.25% 32	25.95% 41	25.95% 41	6.33% 10	158	3.52
Ability to exercise	9.49% 15	14.56% 23	27.22% 43	29.11% 46	19.62% 31	0.00% 0	158	3.35
Ability to volunteer	13.29% 21	15.19% 24	23.42% 37	16.46% 26	16.46% 26	15.19% 24	158	3.09
Ability to conduct household chores	14.56% 23	21.52% 34	31.65% 50	16.46% 26	15.19% 24	0.63% 1	158	2.96
Ability to fulfill family obligations	14.01% 22	24.20% 38	25.48% 40	22.29% 35	11.46% 18	2.55% 4	157	2.93
Ability to spend time with family and friends	17.09% 27	18.35% 29	25.95% 41	20.25% 32	16.46% 26	1.90% 3	158	3.01
Ability to concentrate	9.55% 15	21.66% 34	21.66% 34	25.48% 40	19.75% 31	1.91% 3	157	3.25

**Figure 1—Impact of myeloma**

Respondents (157) were asked, “What is the number one financial implication of your treatment,” and they identified: drug costs (14.65%), lost income due to absence from work (14.65%), and travel costs (10.19%) to be the most significant treatment-related financial implications they had experienced. 11.48% of patients selected ‘other’ and provided explanatory comments, a number of which expressed the desire to select multiple options listed. Here are a few comments provided: “While travel and parking costs have a limited financial implication, the critical financial implication is the costs of drugs. Without provincial coverage of the costs of drugs, they would essentially be inaccessible.”; “Lost great percentage of income from reduced insurances payments”; “My only expenses have been over the counter drugs that I need to control side effects from my myeloma drugs.”

ANSWER CHOICES	RESPONSES
Drug costs	14.65% 23
Travel costs	10.19% 16
Accommodation costs	1.27% 2
Parking costs	9.55% 15
Drug administration fees	0.64% 1
Medical supply costs	1.27% 2
Lost income due to absence from work	14.65% 23
I have had no financial implications related to my myeloma treatment	36.31% 57
Other (please specify)	11.46% 18
TOTAL	157

**Figure 2—Financial implications of myeloma treatment**

4. \*Experiences With Currently Available Treatments—*Subset 1*

When asked if and/or how their myeloma is currently being treated, of 15 respondents in *Subset 1*, 8 indicated they were receiving their third line of therapy, one was on their second line, one was under remission, and one patient’s myeloma had returned but they were not currently under treatment. Four patients did not respond to the question.

ANSWER CHOICES	RESPONSES	
Receiving my second line therapy	9.09%	1
Receiving my third line therapy	72.73%	8
Under remission – my myeloma has not come back	9.09%	1
My myeloma has just come back, and I am about to start a new treatment soon	0.00%	0
My myeloma did come back, and I am not taking any treatment	9.09%	1
I don't know	0.00%	0
<b>TOTAL</b>		<b>11</b>

**Figure 3—Current treatment**

It was inquired of patients (12), “How long have you been, or were previously on the treatment combination of carfilzomib (Kyprolis) and dexamethasone?”; 6 replied they were on the combination for 1–6 months, 4 said 7–12, 2 patients said 2–3 years, and 3 did not answer.

When asked “Have you progressed on lenalidomide (Revlimid) or bortezomib (Velcade) based regimens (or both) in your first-line setting?” 14 (of 15) answered yes, and 1 said no.

In response to the question, “Since taking the treatment combination of carfilzomib (Kyprolis) and dexamethasone, rate on a scale of 1–5 how symptoms associated with myeloma impact or limit your day-to-day activities and quality of life,” patients (12) indicated the most significant impact was upon their ability to work (6), exercise (5), and to conduct household chores (4).

	1 - NOT AT ALL	2 - HAS A LITTLE IMPACT	3 - HAS AN IMPACT	4 - SOMEWHAT OF AN IMPACT	5 - SIGNIFICANT IMPACT	N/A	TOTAL	WEIGHTED AVERAGE
Ability to work	0.00% 0	0.00% 0	16.67% 2	16.67% 2	50.00% 6	16.67% 2	12	4.40
Ability to travel	0.00% 0	16.67% 2	16.67% 2	25.00% 3	16.67% 2	25.00% 3	12	3.56
Ability to exercise	0.00% 0	8.33% 1	16.67% 2	25.00% 3	41.67% 5	8.33% 1	12	4.09
Ability to volunteer	0.00% 0	8.33% 1	16.67% 2	16.67% 2	8.33% 1	50.00% 6	12	3.50
Ability to conduct household chores	0.00% 0	25.00% 3	8.33% 1	25.00% 3	33.33% 4	8.33% 1	12	3.73
Ability to fulfill family obligations	9.09% 1	27.27% 3	9.09% 1	27.27% 3	9.09% 1	18.18% 2	11	3.00
Ability to spend time with family and friends	8.33% 1	16.67% 2	0.00% 0	50.00% 6	8.33% 1	16.67% 2	12	3.40
Ability to concentrate	8.33% 1	16.67% 2	41.67% 5	16.67% 2	8.33% 1	8.33% 1	12	3.00

**Figure 4—Impact on daily life with carfilzomib & dexamethasone**

\* This section presents data from solely from *Subset 1*.

When asked, “How would you rate the common side effects that you have experienced with carfilzomib (Kyprolis) and dexamethasone?” 12 respondents indicated the least tolerable effects to be fatigue (6), and diarrhea (4); while the most tolerable were nausea/vomiting (7) neutropenia (5), and respiratory infections including pneumonia and bronchitis (5).

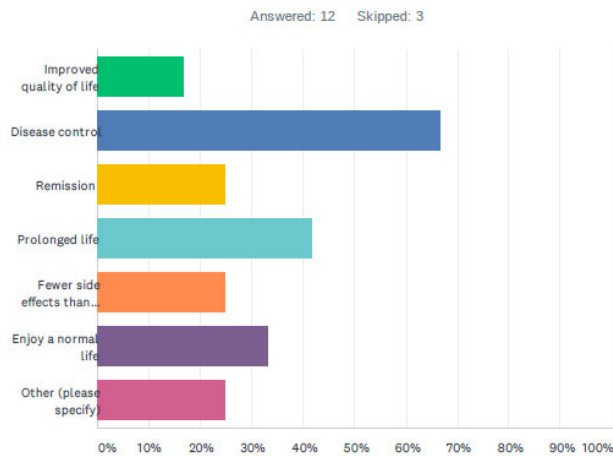
	1 - COMPLETELY INTOLERABLE	2 - SOMEWHAT INTOLERABLE	3 - TOLERABLE	4 - VERY TOLERABLE	5 - EXTREMELY TOLERABLE	N/A	TOTAL
Neutropenia	0.00% 0	8.33% 1	25.00% 3	33.33% 4	8.33% 1	25.00% 3	12
Thrombocytopenia (low levels of platelets)	8.33% 1	8.33% 1	41.67% 5	16.67% 2	0.00% 0	25.00% 3	12
Anemia	8.33% 1	0.00% 0	16.67% 2	33.33% 4	0.00% 0	41.67% 5	12
Cardiac failure	0.00% 0	0.00% 0	8.33% 1	16.67% 2	8.33% 1	66.67% 8	12
Hypertension	0.00% 0	16.67% 2	8.33% 1	25.00% 3	8.33% 1	41.67% 5	12
Respiratory infections, including pneumonia and bronchitis	0.00% 0	0.00% 0	8.33% 1	16.67% 2	25.00% 3	50.00% 6	12
Vein thrombosis	8.33% 1	16.67% 2	0.00% 0	25.00% 3	8.33% 1	41.67% 5	12
Renal failure	9.09% 1	9.09% 1	0.00% 0	9.09% 1	0.00% 0	72.73% 8	11
Ocular disorders	8.33% 1	16.67% 2	8.33% 1	8.33% 1	8.33% 1	50.00% 6	12
Diarrhea	8.33% 1	25.00% 3	16.67% 2	8.33% 1	8.33% 1	33.33% 4	12
Nausea/vomiting	8.33% 1	8.33% 1	0.00% 0	41.67% 5	16.67% 2	25.00% 3	12
Fatigue	25.00% 3	25.00% 3	25.00% 3	8.33% 1	8.33% 1	8.33% 1	12
Shortness of breath, trouble breathing or asthma	8.33% 1	0.00% 0	58.33% 7	25.00% 3	0.00% 0	8.33% 1	12
Infusion-related reaction	0.00% 0	8.33% 1	16.67% 2	25.00% 3	8.33% 1	41.67% 5	12

**Figure 5—Side effects with carfilzomib (Kyprolis) and dexamethasone**

When asked, “Before taking carfilzomib (Kyprolis) with dexamethasone, what were your expectations of the treatment combination? Please rank from 1–7, 1 being the most important, 7 being least important.” Respondents (12) identified remission (3) and prolonged life (3) to be the most important, alongside disease control (2) and fewer side effects than other treatments (2) to a lesser extent.

Respondents (12) then indicated disease control (8), prolonged life (5), and enjoying and normal life (4) to be the expectations their treatment with carfilzomib (Kyprolis) and dexamethasone had best be fulfilled, though their responses show (Figure 6) that to some degree, all listed expectations had been met.

Q16 Which of your expectations has your treatment combination of carfilzomib (Kyprolis) with dexamethasone fulfilled? Please select all that apply.



**Figure 6—Treatment expectations with carfilzomib (Kyprolis) and dexamethasone**

### 5. Improved Outcomes

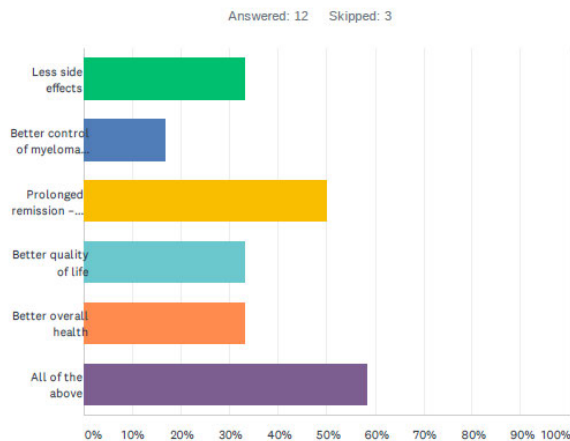
To the question, “If you are taking a drug or were to consider taking a drug for your myeloma, how important is it that it improves your overall quality of life? (Rate on a scale from 1—Not important, to 5—Very important) 68.13% (109) of 160 respondents from all subsets felt it was ‘extremely important’ and 26.25% (42) answered, ‘very important.’

Patients in all subsets were asked, ‘What treatment side effects are most important for you to avoid, please rank from 1 (most important to avoid) to 9 (least important to avoid)’.

- Patients (2) from Subset 3 ranked insomnia (1), neuropathy (1), and pain (1) as ‘most important to avoid.’
- Respondents from Subset 1 (15) indicated fatigue (4), pain (4), and infection (2), to be the most important to avoid.
- Patients from Subset 2 (141) responded that the most important treatment side effects to avoid were infection (42), confusion (20), neuropathy (16) and pain (16).

When asked, ‘If you were eligible to receive the isatuximab (Sarclisa; for injection) with carfilzomib (Kyprolis) and dexamethasone, what do you believe the benefits would be for you, compared to the type of treatment you are currently receiving, or have already received?’ subset 1 respondents (12) expected to receive all benefits listed (7), and expected ‘prolonged remission—where myeloma is not present’ (6).

Q18 If you were eligible to receive the isatuximab (Sarclisa; for injection) with carfilzomib (Kyprolis) and dexamethasone, what do you believe the benefits would be for you, compared to the type of treatment you are currently receiving, or have already received?



**Figure 7—Perceived benefits of the treatment under review**

When asked, ‘If you were eligible to receive isatuximab (Sarclisa; for injection) with carfilzomib (Kyprolis) and dexamethasone, what do you think the disadvantages would be for you (compared to the type of treatment you are currently receiving, or have already received)?’ 8 respondents from Subset 1(12) answered, and thought these disadvantages would be, increased fatigue (4), decreased physical abilities (3), and difficulty of treatment (travel, injection time, etc.) (3). When asked, ‘Do you feel that receiving isatuximab (Sarclisa; for injection) with carfilzomib (Kyprolis) and dexamethasone to treat your myeloma could improve your health and well-being?’ among patients from Subset 1 (12) 3 said ‘yes,’ and 9 said they ‘did not know.’ No respondent said no.

Included in the survey for all 3 patient subsets, was the open-ended question, ‘What is important to you when it comes to treating your myeloma?’. Respondents most frequently mentioned effectiveness of treatment, quality of life, and achieving a long remission to be important to them. Some other responses of relevance are as follows: ‘Ability to access treatment without any payment other than my OHIP card’; ‘Access to latest technology’; ‘To have a lasting remission and not spend too much time in hospital for treatment.’; ‘Provide the ability to lead a nearly normal life with a minimal frequency of visits to doctors and the hospital’; ‘I would like to be well informed about consequences of treatment and be able to continue with a relatively decent quality of life while undergoing treatment.’; ‘Quality of life, how I feel during treatment and if I’m able to be with my family and friends. How long treatment will work.’

**6. \*Experience with Drug Under Review—Subset 3**

When asked to rate the effectiveness of isatuximab (Sarclisa) combined with carfilzomib (Kyprolis) and dexamethasone in controlling their myeloma, on a scale from 1 ‘Not effective’ to 5 ‘Extremely effective,’ one respondent felt it was ‘5—extremely effective’.

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\* This section presents results from Subset 3.



To the question, “Did the administration of isatuximab (Sarclisa; for injection) (i.e., the way you took the treatment, by injection) have a negative effect on you?” one respondent indicated it did not have a negative effect upon them.

When it was enquired “Based on your personal experience with the combination of isatuximab (Sarclisa; for injection) with carfilzomib (Kyprolis) and dexamethasone, how would you rate the overall side effects? 1 is “Completely intolerable” and 5 is “Extremely tolerable”” one respondent replied “3—Tolerable.”

When asked, “How would you rate (on a scale from 1—Completely intolerable to 5—Extremely tolerable), the common side effects you have experienced with isatuximab (Sarclisa) in combination with carfilzomib (Kyprolis) and dexamethasone?” one respondent indicated that nausea/vomiting (1), and thrombocytopenia (1) were “completely intolerable.”

To the question “Did your myeloma treatment of isatuximab (Sarclisa) in combination with carfilzomib (Kyprolis) and dexamethasone improve your health and well-being” one respondent replied “yes.”

When asked “How would you rate your quality of life with isatuximab (Sarclisa) in combination with carfilzomib (Kyprolis) and dexamethasone’, on a scale from 1–5 (1 being “poor quality of life” and 5 being “excellent quality of life”)?”, one respondent said “4—very good.”

To the question “Did your myeloma treatment combination of isatuximab (Sarclisa) in combination with carfilzomib (Kyprolis) and dexamethasone improve your long-term health outlook,” one respondent said “yes.” When asked if isatuximab (Sarclisa) with carfilzomib (Kyprolis) and dexamethasone had met their expectations in treating their myeloma, one respondent said “yes.”

## 7. Anything Else?

Across subsets, when the opportunity was provided for patient comments, their responses frequently echoed similar sentiments. These are: desiring treatments to have **maximum effectiveness** but with **non-debilitating side effects**; to be **minimally occupying** their time with numerous visits to the hospital, and to ultimately achieve the **longest remission possible** for themselves (in lieu of a cure); all of which contribute to their (the patients’) abilities to lead a “**normal**” life (one of *good quality*). Patients also stated that receiving **information** and explanation about new and emerging treatments was important to them, as well as the timely provision of **access** to these treatments through government approval and coverage. These concerns are of particular significance considering (as detailed in section 4) the majority of Subset 1 patients (9 of 12 respondents) answered ‘I don’t know’ when asked if they would benefit from isatuximab (Sarclisa) in combination with carfilzomib (Kyprolis) and dexamethasone. Patients need a better understanding of their treatment options, and treatment sequencing, and they know it. There is no such thing as “one-treatment-fits-all” when it comes to treating myeloma. What works for one patient may not work for another, which is why each case must be assessed individually. As more and more treatment becomes available, it is important for patients to be able to chart, with their healthcare provider, the best action plan for them. This cannot be done without understanding their treatment options and their effects on their quality of life.

## Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH reimbursement review process, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it. **No**
2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it. **No**
3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

Company	Check Appropriate Dollar Range			
	\$0 to 5,000	\$5,001 to 10,000	\$10,001 to 50,000	In Excess of \$50,000
Amgen Canada Inc.				X
Sanofi				X
Janssen				X
Bristol-Myers Squibb Canada Co.				X
Celgene				X
Takeda Canada Inc.				X
Merck Canada Inc.			X	
Pfizer Canada			X	
Karyopharm Therapeutics				X
Novartis	X			
GlaxoSmithKline Inc.			X	
Leo Pharma Inc.		X		
Rapid Novor Inc.			X	

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name: Martine Elias  
 Position: Executive Director  
 Patient Group: Myeloma Canada  
 Date: July 17, 2021