

CADTH COMMON DRUG REVIEW

Patient Input

idecabtagene vicleucel (Brand Name Confidential)

(Celgene Inc., a Bristol Myers Squibb company)

Indication: Multiple myeloma

CADTH received patient input from:
Myeloma Canada

January 15, 2021

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CADTH Reimbursement Review

Myeloma Canada Patient Input Regarding idecabtagene vicleucel

Name of the Drug and Indication	Idecabtagene vicleucel
Name of the Patient Group	Myeloma Canada
Author of the Submission	[REDACTED]
Name of the Primary Contact for This Submission	[REDACTED]
Email	[REDACTED]
Telephone Number	[REDACTED]

1. About Your Patient Group

See registration information and visit our website at www.myeloma.ca.

2. Information Gathering

Myeloma Canada conducted a patient survey via email and through social media from December 17, 2020, to January 4, 2021. The survey gathered feedback about **idecabtagene vicleucel**. A total of 388 individuals with myeloma responded to the survey. From this total they were divided into 2 sets of myeloma patients:

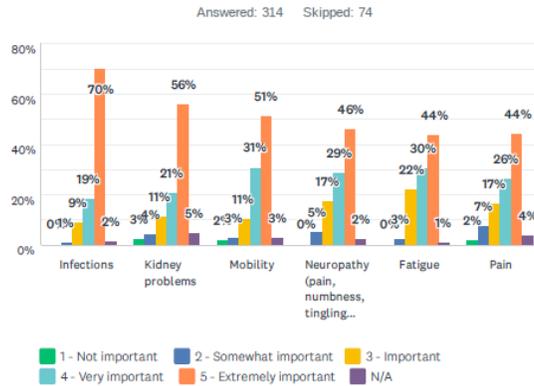
- **Subset 1** — A total of 161 did not have the treatment under review. However they did qualify to receive the treatment based on previous therapy regimens. These respondents were from across Canada as follows: ON 43%, BC 20%, AB 13%, QC 12%, SK 3%, MB 2%, NB 2%, NF 2%, NS 2%, PE 1%.
- **Subset 2** — A total of 14 respondents had received a CAR T cell therapy. The 14 respondents were from ON 36%, AB 21%, BC 21%, QC 14% and SK 7%.

3. Disease Experience

Graphic 1—Control of myeloma

When asked to rate on a scale of 1–5 (1 is "Not important", 5 is "Very important") how important it is for them to control various aspects of their myeloma, 70% of the respondents (314) indicated that their major concern was control of infections, followed kidney problems (56%) and mobility (51%).

Q4 Please rate on a scale of 1 - 5, how important it is for you to control various aspects of your myeloma. 1 is "Not important", 5 is "Extremely important".



* Please note the complete text for neuropathy is "Neuropathy (pain, numbness, tingling, swelling or muscle weakness)".

Graphic 2—Daily life impact

When asked, "Please rate on a scale of 1–5, how much symptoms associated with myeloma impact or limit your day-to-day activities and quality of life (1 is 'Not at all', and 5 is 'Significant impact')", respondents (315) indicated it significantly impacted on their ability to travel, to work and to exercise.

	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 travel	8.65% 27	13.46% 42	22.44% 70	17.95% 56	34.94% 109	2.56% 8	312	3.59
Ability to work	9.58% 30	15.97% 50	17.57% 55	12.78% 40	26.20% 82	17.89% 56	313	3.37
Ability to exercise	7.42% 23	19.68% 61	26.77% 83	25.16% 78	20.97% 65	0.00% 0	310	3.33
Ability to conduct volunteer activities	9.87% 31	16.88% 53	22.61% 71	18.15% 57	18.47% 58	14.01% 44	314	3.21
Ability to conduct household chores	11.46% 36	20.70% 65	27.07% 85	23.25% 73	16.88% 53	0.64% 2	314	3.13
Ability to concentrate	12.54% 39	22.19% 69	22.83% 71	23.15% 72	18.33% 57	0.96% 3	311	3.13
Ability to spend time with family and friends	16.35% 51	19.23% 60	26.60% 83	16.99% 53	19.23% 60	1.60% 5	312	3.04
Ability to fulfill family obligations	13.78% 43	21.47% 67	30.77% 96	17.31% 54	14.74% 46	1.92% 6	312	2.98

4. Experiences With Currently Available Treatments

When asked, "If you are taking a treatment or were to consider taking treatment for your myeloma, how important is it to improve your overall quality of life? Rate on a scale of 1–5, 1 is 'Not important' and 5 is 'Extremely important'," 61% of the respondents (288) expressed that it is extremely important, 30% felt it was very important and 9% selected important.

When asked "If you are receiving a treatment for your myeloma at the moment, please indicate how often you need to visit a cancer centre," 40% of the respondents (315) visit a cancer centre once a month.

Graphic 3—Financial implications

When asked, “What is the most significant financial implication of your treatment? If there is more than one implication, please check all that apply,” respondents (313) identified drug costs (35%), parking costs (33%), lost income due to absence from work (29%) as their most significant financial implications.

ANSWER CHOICES	RESPONSES	
I have had no financial implications related to my myeloma treatment	35%	110
Drug costs	33%	102
Parking costs	29%	90
Lost income due to absence from work	25%	78
Travel costs	22%	70
Other (please specify)	10%	31
Medical supply costs	8%	26
Accommodation costs	7%	22
Drug administration fees	7%	21
Total Respondents: 313		

**Under “other” (not reflected from the list above) respondents added additional responses: “disability mental health—costs”, and “had to retire.”*

When asked, “Do you need the support of a family member or caregiver to help you manage your myeloma or your treatment-related symptoms?” 54% of the respondents (314) answered “yes”.

When asked “What is most important to you when it comes to treating your myeloma,” the respondents (266) identified effective treatments, fewer side effects, and better quality of life as what mattered most to them. Here are a few comments shared by respondents: “Efficacy of the treatment for improved prognosis and ability to maintain similar quality and function of life activities”; “To get the best available treatment out there”; “Extending my life and having hope for treatments going forward and being pain free”; “I would like to get off medications and as close to a long remission and cure as possible as I am a young myeloma patient.”; “It’s important that I am able to receive the most up to date treatment and have access to the best minds in the field. Considerations for my mental health and how best to maintain a positive outlook is also very important.”; “Being able to have a lifestyle rather than just a world revolving around treatment”; “Quality of life above all with minimal side effects.”; “Minimizing side effects and portability or reduced frequency of time in hospital”; “Ability to cope with side effects”; “Accessibility to the newest and most effective treatment with minimal side effects.”

5. Improved Outcomes (Subset 1)

In this section, we will present results from the subset 1 of the respondents.

To the question "Please answer the following statement: I have received at least three prior therapies that included an (IMiDs), such as thalidomide, or lenalidomide (Revlimid), or pomalidomide (Pomalyst) and / or a proteasome inhibitor (PI) such as bortezomib (Velcade) or carfilzomib (Kyprolis) or ixazomib (Ninlaro) and an anti-CD38 antibody, either daratumumab (Darzalex) or isatuximab (Sarclisa).", 161 of the respondents (297) answered “yes”.

Graphic 4—Understanding CAR T

We asked if they ever heard of CAR T cell immunotherapy to subset 1 respondents: 73% (117) of them answered “yes”, 23% answered “no”, and 4% did not know what was CAR T.

They were asked to select the statement that best fit their understanding of CAR T cell immunotherapy. 57% of the respondents (161) answered correctly when they selected “all of the above”. We interpret this as having more than half of the participants understand the process involved in this therapy and that it is different from what they are used to.

ANSWER CHOICES	RESPONSES	
All of the above	56%	90
I don't know	22%	36
T cells (T lymphocytes, which are special types of white blood cells) are first harvested from a cancer patient and then modified in a lab before they are reintroduced into the patient's body intravenously	11%	18
CAR T cell immunotherapy involves genetically modified cells responsible for killing cancer cells	6%	10
CAR T cell immunotherapy is a new type of blood cancer treatment	4%	7
TOTAL		161

Graphic 5—CAR T benefits

When asked what they believe are the benefits of the CAR T therapy for them, compared to the type of treatment they are currently receiving, or have already received, the majority (75%) of respondents (118) believed they would receive all of the benefits listed.

ANSWER CHOICES	RESPONSES	
All of the above	75%	118
Prolonged remission – where my myeloma is not present	34%	53
Not having to take a myeloma treatment because my myeloma is under remission	27%	43
Better quality of life	24%	37
Better overall health	21%	33
Better control of myeloma symptoms	19%	30
Less side effects	16%	25
None of the above	1%	2
Total Respondents: 157		

Respondents (155) were asked “Considering that treatment options are becoming limited at this point in your myeloma journey, other than participating in clinical trials or another treatment, indicate what an estimated prolonged remission of 34.2 months means to you at this stage in your life.” A prolonged remission of 34.2 months was “extremely desirable” to 89 respondents, “very desirable” to 19 respondents, “desirable” to 16 respondents, “somewhat desirable” to 4 respondents, and 25 respondents didn’t know what it meant to them.

Graphic 6—Understanding risks

We explained the treatment procedures and risks to the respondents as follows: “In some cases, the patient’s myeloma can progress during this time (i.e., the myeloma comes back) and the patient may require an additional (bridging) therapy. If this happens the patient may no longer be eligible to proceed to the next step which is to receive their modified T-cells. In this case other treatment options are available and will be presented to the patient.”

We asked them to indicate the sentence that best reflects their sentiments about this kind of situation, 46% (72) of the 156 respondents said they were not sure about how it makes them feel and would like

to discuss this situation further with my hematologist/oncologist. 23% (36) were willing to take the risk that their myeloma might come back.

ANSWER CHOICES	RESPONSES	
I am willing to take the risk that my myeloma comes back while I wait for my T-cells to be reintroduced	23%	36
I am concerned about my myeloma coming back but I can live with waiting for my T-cells to be reintroduced	24%	37
I am overly concerned about the possibility of my myeloma coming back and I am not willing to take this risk	3%	4
I am not sure how this makes me feel and would like to discuss this situation further with my hematologist/oncologist	46%	72
Other (please specify)	4%	7
TOTAL		156

Graphic 7—Concerns about side effects

When asked "Which CAR T treatment side effects listed below are most concerning to you? On a scale of 1 (most troublesome) to 5 (least troublesome), please rate each of the following statements.", the most concerning side effects for the respondents (150) were loss / decrease of cognitive abilities (66%) and loss of physical abilities (and mobility) (54%).

	1 - MOST TROUBLESOME	2 - A LITTLE TROUBLESOME	3 - TROUBLESOME	4 - SOMEWHAT TROUBLESOME	5 - LEAST TROUBLESOME	N/A	TOTAL
Loss / decrease of cognitive abilities	66% 97	14% 20	15% 22	4% 6	1% 2	1% 1	148
Loss of physical abilities (and mobility)	54% 80	20% 30	22% 32	3% 5	1% 1	0% 0	148
Anemia	24% 36	26% 38	33% 48	14% 20	3% 5	0% 0	147
Neutropenia (low white blood cell count leading to repeated infections)	37% 55	22% 32	28% 41	9% 14	4% 6	0% 0	148
Thrombocytopenia (bleeding into the skin and bruising)	21% 31	26% 38	30% 44	16% 23	7% 11	0% 0	147
Cytokine Release Syndrome (severe flu-like symptoms - fever, nausea, headache, rapid heartbeat, low blood pressure, trouble breathing, rash)	55% 81	16% 23	18% 26	8% 12	3% 4	1% 1	147
Fatigue	20% 29	30% 44	31% 45	17% 25	2% 3	0% 0	146
Neuropathy (numbness, tingling, edema or muscle weakness)	24% 35	32% 47	26% 38	15% 22	3% 5	0% 0	147
Pain	25% 37	32% 47	30% 45	11% 16	3% 4	0% 0	149
Muscle weakness	21% 31	39% 57	28% 42	8% 12	4% 6	0% 0	148
Typical cold symptoms (upper respiratory tract infection)	21% 31	29% 43	28% 41	13% 19	9% 14	0% 0	148

Respondents (151) were asked if they felt CAR T cell immunotherapy to treat their myeloma could improve their health and well-being: 48% answered “yes”, 4% answered “no”, and 48% did not know.

Respondents (150) were asked if they felt CAR T cell immunotherapy to treat their myeloma could improve their long-term health outlook: 58% answered “yes”, 3% said “no”, and 39% did not know.

When asked “With what you know today, what treatment option would you consider first as your next treatment?”, among the 151 respondents 35% would choose CAR T cell immunotherapy, 15% would choose another treatment suggested to them, 9% would choose a clinical trial, and 42% don’t know what they would choose.

A total of 48 respondents provided comments. Here are a few comments shared by respondents: “The potential of extending my life for shy of three years is much better than going through clinical trials that are only effective on average for 9 months”; “The patient and caregiver are under a lot of stress try to adjust. Adjusting to travel, find accommodation, finding your way to the hospital, new doctors, having to go to more than one hospital. Unknown side affects. More medical expenses.”; “3 & ½ yrs is not that long”; “I have been in four clinical trials. I would try another before I would consider CAR T cell immunotherapy if I were eligible. I am 80 years old.”

6. Experience With Drug Under Review (Subset 2)

In this section, we will present results from the subset 2 of the respondents.

To the question “Have you been treated or are you in the process of receiving a CAR T cell immunotherapy to treat your myeloma?”, 14 of the total number of respondents (314) answered “yes”. These respondents were asked which CART had they received. Only 1 received the treatment under review, 5 answered they had received another CAR T cell therapy, and 8 didn’t know which CAR T cell therapy they received. We made the decision to present the data on all the patients who had received a CAR T and answered the questions. This gives us a directional perspective of this new therapy and will serve the pCODR committee to better understand overall patient experience.

When asked “How long ago you received your idecabtagene vicleucel (ide-cel, bb2121) CAR T therapy?”, 1 respondent answered “less than 3 months ago”, 2 answered “between 3 and 6 months ago”, 1 answered “between 6 and 12 months ago”, 1 answered “over a year ago”, and 1 answered “over 2 or more years ago”.

When asked “Based on your experience of the idecabtagene vicleucel (ide-cel, bb2121) CAR T cell immunotherapy, how would you rate the effectiveness of this treatment in helping to control your myeloma?”, among the 6 respondents, 1 answered it was “extremely effective”, 3 answered “very effective”, 1 answered “effective”, and 1 answered “not effective”.

When asked “Based on your experience with the idecabtagene vicleucel (ide-cel, bb2121) CAR T immunotherapy, how would you rate the overall side effects?”, 3 out of 6 respondents answered is was “very tolerable”, 2 answered it was “tolerable”, and 1 answered it was “somewhat tolerable”.

Graphic 8—Side effects of the treatment under review

When asked “Amongst the most frequent side effects seen in patients who participated in the study of the idecabtagene vicleucel (ide-cel, bb2121) CAR T cell immunotherapy, which ones have you experienced? Please select all that apply and rate the side effects you have experienced. 1 being ‘Totally unbearable’ and 5 ‘Extremely bearable’,” neutropenia (67%) and decrease of cognitive abilities (67%) were the more bearable side effects for the 6 respondents.

	1 - TOTALLY UNBEARABLE	2 - VERY UNBEARABLE	3 - BEARABLE	4 - SOMEWHAT BEARABLE	5 - EXTREMELY BEARABLE	N/A	TOTAL RESPONDENTS
Decrease of cognitive abilities	0% 0	0% 0	17% 1	0% 0	67% 4	17% 1	6
Loss of physical abilities (and mobility)	0% 0	0% 0	17% 1	17% 1	50% 3	17% 1	6
Anemia	0% 0	0% 0	33% 2	0% 0	33% 2	33% 2	6
Neutropenia (low white blood cell count leading to repeated infections)	0% 0	0% 0	17% 1	17% 1	67% 4	0% 0	6
Thrombocytopenia (bleeding into the skin and bruising)	0% 0	0% 0	0% 0	0% 0	33% 2	67% 4	6
Cytokine Release Syndrome (severe flu-like symptoms - fever, nausea, headache, rapid heartbeat, low blood pressure, trouble breathing, rash)	0% 0	0% 0	50% 3	17% 1	33% 2	17% 1	6
Fatigue	0% 0	0% 0	50% 3	17% 1	33% 2	0% 0	6
Neuropathy (numbness, tingling, edema or muscle weakness)	0% 0	0% 0	0% 0	0% 0	50% 3	50% 3	6
Pain	0% 0	0% 0	0% 0	17% 1	50% 3	33% 2	6
Muscle weakness	0% 0	0% 0	17% 1	17% 1	50% 3	17% 1	6
Typical cold symptoms (upper respiratory tract infection)	0% 0	0% 0	0% 0	0% 0	50% 3	50% 3	6

When asked "You were most likely not on a treatment for your myeloma after your idecabtagene vicleucel (ide-cel, bb2121) CAR T cell immunotherapy either for a prolonged or indeterminate period of time. How important was this treatment interruption for you?", 4 out of 6 respondents answered it was "extremely important", 1 answered "somewhat important", and 1 answered "not important".

To the question "Did the CAR T therapy idecabtagene vicleucel (ide-cel, bb2121) meet your expectations in treating your myeloma?" 5 out of the 6 respondents answered "yes" and 1 answered "no." Four comments were added to this question: "I am so far in very very good remission"; "I was in remission for about a year"; "Didn't get depth of response as we were hoping."; "No more chemo!"

When asked "Did the CAR T therapy idecabtagene vicleucel (ide-cel, bb2121) improve your long-term health outcomes?," all 6 respondents answered "yes". Two respondents provided comments: "Yes, I'm still alive!"; "I would otherwise have run out of options, but more are now available."

When asked where there are currently regarding this treatment, 3 out of 6 respondents selected "Still under remission—my myeloma has not come back," 1 selected "My myeloma has just come back, and I am about to start a new treatment soon," and 2 selected "My myeloma did come back, and I am currently under a new treatment."

When asked in an open-ended question if there was anything else about their experience with the treatment under review they would like to share, 2 out of 6 respondents provided comments: "The required 2 weeks in hospital seemed very very long"; "I had it done 2x and it gave me drug free time. I would do it again if they would allow me."

7. Anything Else?

The effectiveness of a treatment is what most of myeloma patients are looking for besides limited side effects and having a good quality of life. Given there is no single treatment effective for all myeloma patients, various treatment options must be available to improve patients' prognosis and quality of life. Myeloma is what is known as a relapsing-remitting cancer; it alternates between periods of symptoms and/or complications that need to be treated and periods of remission that do not require treatment. Providing patients with an effective treatment and giving them a prolonged remission with limited side effects can play a major role in their well-being and lead them to live a full and productive life. As we can glean from the patients' responses, a CAR T therapy can represent an important benefit for them despite the number and severity of side effects. Even though a small number of them were actually on the treatment under review, about a half of the respondents fit the criteria's. For these patients treatment options are becoming limited at this point in their myeloma journey. The reality is their lives depend on treatment options, because to date there is no cure for myeloma.

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. **Yes. We hired Colucci Heath to summarize the data for this report.**
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: January 13, 2021