

CADTH COMMON DRUG REVIEW

Patient Group Input Submissions

abobotulinumtoxinA (Dysport Therapeutic)

(Ipsen Biopharmaceuticals Canada, Inc.)

Indication: To reduce the subjective symptoms and objective signs of cervical dystonia (spasmodic torticollis) in adults

Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.

Dystonia Medical Research Foundation Canada — permission granted to post.

CADTH received patient group input for this review on or before February 22, 2017.

CADTH posts all patient input submissions to the Common Drug Review received on or after February 1, 2014 for which permission has been given by the submitter.

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.

Dystonia Medical Research Foundation Canada

General Information

Name of the drug CADTH is reviewing and indication(s) of interest	Dysport (abobotulinumtoxinA)
Name of the patient group	Dystonia Medical Research Foundation Canada
Name of the primary contact for this submission:	[REDACTED]
Position or title with patient group	[REDACTED]
Email	[REDACTED]
Telephone number(s)	[REDACTED]
Patient group's contact information:	
Email	info@dystoniacanada.org
Telephone	416-488-6974
Address	121 Richmond Street West, Suite 305, Toronto, ON M5H 2K1
Website	www.dystonaicanada.org
Permission is granted to post this submission	Yes

Submitting Organization

The Dystonia Medical Research Foundation was founded in 1976 by Samuel and Frances Belzberg of Vancouver, after their daughter was diagnosed with generalized dystonia. The mission of the Dystonia Medical Research Foundation Canada (DMRFC) is to advance research for more treatments and ultimately a cure; to promote awareness and education; and to support the needs and well being of affected individuals and families. DMRFC is a registered non-profit Canadian charity governed by a volunteer [Board of Directors](#). As we are a charitable organization dedicated to dystonia research, education and awareness, we represent the needs of the dystonia patient population in Canada.

Conflict of Interest Declarations

- a) *We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:*

As a charitable organization, we receive support from a variety of places. In 2016, we received financial support from the following pharmaceutical companies: Allergan, Merz, Ipsen. These funds are for a number of initiatives including: patient educational sessions, the development of a patient package (to be distributed to movement disorder clinics across the country), brochures, and event sponsorships.

- b) *We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:*

None

Condition and Current Therapy Information

Information Gathering

We launched an online survey on January 23, 2017 that ran through February 9, 2017. The DMRF Canada promoted the online survey by e-communications sent out on January 24, 2017 to select patients who we know have Cervical Dystonia. As well, an email was sent out to approximately 30 Volunteer Support Group Leaders from across the country (with an ask to share the survey with members of their group. The survey was also posted to the DMRF Canada Facebook page and on the DMRF Canada Twitter page. One reminder message was posted halfway through the survey campaign. **In total, we had 80 people participate (in whole or in part) in the Patient survey.**

Information about the caregiver experience in Canada was obtained through an online survey as well. We launched a separate online survey to caregivers on January 23, 2017 that ran through February 9, 2017. We promoted this online survey by the same methods as outlined above. **In total, we had 3 people participate (in whole or in part) in the Caregiver survey.**

The following themes emerged from our survey:

1. Cervical Dystonia patients experience both physical and emotional distress as a result of their condition;
2. The condition (Cervical Dystonia) has a profound impact on daily life (including physical, social interactions, financial, and work options);
3. Although there are a number of treatment options available, the overall satisfaction rate of current treatment options is relatively low (see question 6 for more details);
4. Current treatments are also not always easily accessible, either due to cost/financial hardship, availability of specialists, or travel challenges;

Impact of Condition on Patients

The following questions were asked of **PATIENTS** (see Appendix 1 for the complete table of answers to each question):

Question 1: What aspects of cervical dystonia are most important to control? Check all that apply.

The most common answer choice was “Neck Pain”, with 84.4% of respondents identifying this.

Note: 18 of the 77 respondents included “Other” responses. Many of those responses mirrored the options listed above. In addition to the main choices listed above, patients also included: “twisting of torso”, “head movement – shaking and twisting”, and “tremors” as additional aspects that need to be controlled.

Question 2: How do ongoing symptoms affect your day-to-day life? Check all that apply.

The two most common answer choices were: “Limit you from participating in leisure activities and socializing” and “Affect you emotionally”, both with 71.4% of respondents choosing this answer.

Note: 16 of the 77 respondents included “Other” responses. The most common “Other” responses focused on the impact in “all aspects of my daily life”.

Question 3: What limitations have you experienced because of having cervical dystonia? Check all that apply.

The most common limitation listed was “Unable to be physically active” with 68.6% of respondents answering this question. 57.1% of respondents said that they are unable to work as a result of their dystonia.

Note: 22 of the 70 respondents who answered this question selected “Other”, but many of these responses were left blank. A few of them focused on the fact that cervical dystonia has a number of limitations – one quote in particular highlights the many areas of life that are impacted:

“lost self confidence/self esteem, depression, difficulty to function & do daily simple tasks like eating n enjoying a meal, doing some house cleaning, study for a course or going back to school, difficulty to drive to check turn neck to check blind spot view, being a proactive and active citizen”

Question 4: Since diagnosis, which of the following therapies have you used to treat your dystonia?

The most common treatment was BOTOX, with 85.3% of patients having tried it. The second most common treatment was oral medications, with 65.3% of patients having tried some form of oral treatment.

Note: 30 of the 75 respondents selected “Other”. The most common responses in the “Other” category were: Physiotherapy (with approximately 10 responses) and acupuncture (with approximately 10 responses) - in some cases, patients included both methods. Over 10 other treatment methods were named in the “Other” category, including yoga for dystonia, dry cupping, medical marijuana, etc. This clearly highlights the fact that patients are not satisfied with the treatments that they have tried to date, and that they continue to look for new therapies.

Question 5: Which of the following therapies are you using currently. Check all that apply.

64.8% of patients are using BOTOX currently, followed by 45.9% of patients using some form of oral medication. 23 of 74 patients selected the “Other” category and again, the most common answer in this category was Physiotherapy.

Question 6: How effective is your current therapy at controlling the following symptoms of cervical dystonia we got the following responses?

Only up to 6.15 % of patients felt that the current medication they were using was “excellent” in managing any of their symptoms (for those with headaches). Up to 20.55% found the current medication they were using was “very good” in managing any of their symptoms (Neck Pain).

Of those surveyed, when looking at the various symptoms of cervical dystonia, between 27.69% and 46.27% individuals said that the current therapy controlled their symptom “just okay” or “not effective”.

Question 7: Are you currently taking a neurotoxin (BOTOX, Xeomin, Dysport) to treat cervical dystonia?

62% of patients surveyed are currently using BOTOX. An additional 12.99% are using Xeomin. 24.6% are not currently using a neurotoxin.

Question 8: Which of the following adverse effects have you experienced from the neurotoxin you use to treat cervical dystonia? Check all that apply.

Patients highlighted some of the adverse effects experienced with their current neurotoxin, the most frequent side effect being both “Muscle weakness near injection area” (39.29%) and “Headache, muscle stiffness, neck or back pain” (39.29%).

Question 9: Have you had any difficulty accessing therapies for cervical dystonia (i.e. financial challenges, travel limitations, supply issues)? Check all that apply.

Overwhelmingly (59%), respondents stated that they experience financial challenges as a result of their cervical dystonia therapy. This was followed by those who said that they were unable to travel to the site to receive treatment (20.41%). The majority of the responses received in the “Other” category concerned the same three issues:

Financial:

“Challenges with the insurance company approving long term disability”

“Physiotherapy, chiropractor and massage therapy are not all covered enough for the year. I have to supplement from my own money. I live on CPP and OAP.”

Travel/Limited Access to Treatment:

- “I require BOTOX treatment every three months. Since there is only 1 physician providing this service, sometimes I have to wait 4 months”
- “On 5 month wait list to see neurologist for Botox injection”

- “The treatment centre is a fair distance, more than 100 kilometres”
- “I have to travel to Toronto from Sudbury every 3 months”

Impact on Caregivers

The following questions were asked of **CAREGIVERS** (see Appendix 2 for the complete table of answers to each question):

Question 1: What challenges do caregivers face in caring for someone with cervical dystonia? Check all that apply.

The most frequent two responses were: Access to speciality physicians/movement disorder specialists, and Demands on Personal Time.

Question 2: How do current treatments impact the role of the caregiver? Check all that apply.

Two of the three survey participants responded as follows (below). In addition, one of the caregivers included the following comment in the “Other” category: *“We do not have a Dystonia specialist we rely on a Neurologist”*.

Question 3: Are there challenges for caregivers in dealing with the adverse effects related to the current therapy/therapies a loved one is taking? Check all that apply.

Two respondents provided their feedback in the “Other” category with the following answers: “heavily medicated” and “still waiting for specialist appointment”

Information about the Drug Being Reviewed

Information Gathering

To the best of our knowledge, there are no patients residing in Canada who are currently taking Dysport for cervical dystonia. However, telephone interviews were conducted with two patients. One of these patients uses Dysport and resides in the USA. The other interview was with the caregiver of a woman who currently resides in BC, but who used to receive Dysport when she lived in Holland.

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

Interview #1: Patient living in the US who is currently using Dysport. The telephone interview with this individual took place on February 9, 2017

The patient was diagnosed with Cervical Dystonia in Jan 2004. His symptoms were tremors, which graduated to neck pulling, eventually with his chin pulling over to his left shoulder. “I got to the point the I was stuck looking over my left shoulder”. This patient was treated with a number of different medications (oral) that had little effect. Eventually, the patient moved to Botox and he started seeing an osteopath in Toronto when he travelled there for work. He says this “helped to me to get my life back on track – probably a year and a half after originally being diagnosed”. This patient has been using a neurotoxin ever since that time.

The patient eventually switched from Botox to Dysport. When asked why he made the switch he said, “I switched to a doctor that was a local expert in movement disorders”. The patient went on to say: “He (the physician) said that he was most comfortable injecting Dysport, so he suggested it. I had never heard of it. I made the switch a year and a half ago in 2015”.

When asked if there was any real difference between the two toxins, from his perspective, this patient said: “I used to have trouble swallowing – the toxin would leak from the neck muscles with Botox, for the first two weeks or month after injection. That was only in the higher doses, which I hadn’t taken that high dose since 2006, 2007”. The patient said that with Dysport, he has not experienced trouble swallowing.

When asked about his experience with Dysport, and the positive or negative effects of being on this drug, the patient provided the following information:

- Reduction in Headache: “I don’t have headaches”, so not applicable (NA).
- Neck Pain: “Yes, it helps with the stiffness”

- Fatigue – “I don’t feel fatigue”, so NA.
- Neck Twisting: “Yes, it helps”
- Difficulty Swallowing: “Not an issue”, so NA.
- Emotional Distress: “Not an issue”, so NA.

This patient currently receives an injection every 13 weeks. When asked about this experience he said: “It takes about a week before it kicks in and then it starts to wear off at about two months. By 13 weeks, I’m ready for another shot. I find it lasts longer than the Botox. That’s what the doctor said would happen, too”.

When asked the question: “Is Dysport better or worse at managing your Cervical Dystonia symptoms”, the patient said: “I would say it helps more – I’ve felt better since I’ve taken it. Its not as bad when the 13 weeks are up”.

Interview #2: The husband of a patient living in Canada who used to use Dysport when she lived in Holland. The telephone interview with this individual took place on February 10, 2017.

When I asked the caregiver to tell me about living with Cervical Dystonia he explained that his wife used to use Dysport five years ago, when they were living in Europe. She is now using Xeomin. He said that when they came to Canada in June 3, 2012, the doctor that she found recommended Xeomin.

When I asked the patient if he could recall any difference or change that his wife experienced with Dysport vs. Xeomin, he said: “With Dysport, she gets only one injection in the muscle, but now they find that they have to use more Xeomin than Dysport. So, she gets more injections of Xeomin”. When I asked him if he thought his wife had a preference between Dysport vs. Xeomin, he said perhaps, but he wasn’t sure. His explanation was that the two drugs were injected a bit differently, and because he said she gets more needles in her body now than she did before, she may be interested in changing to Dysport. It would depend on if it was accessible and the cost.

When I asked him about the efficacy between the two drugs, he confirmed that the thought the efficacy was the same with both, but repeated that there are more needles required with Xeomin than there were for Dysport.

He was interested in learning more if the drug becomes accessible in Canada. “We could always try it out. If Dysport is coming here, we should know that. We would like to know”.

Additional Information

We also conducted a telephone interview with an individual who does not use Dysport, but who resides in a more remote area in northern Ontario in order to get a sense of how difficult it is to get treatment for her Cervical Dystonia. This telephone interview took place on January 26, 2017.

For her, and the other members of the Dystonia Community in her area, the biggest issue is that there is no specialist who is able to inject them. She drives or flies to Toronto every three weeks. She said that she is lucky, because she has the time and resources available to her to do this (she also has family in Toronto), but she said there other others in her group, particularly those who are aging, who are not in a position where they are willing to make the trip anymore. Her final comment was: “We just really need some more support in Sudbury. It’s really not realistic to expect patients to travel that kind of distance to get treatment”.

Appendix 1: Patients

Question 1: What aspects of cervical dystonia are most important to control? Check all that apply.

Answer Choices	Responses	
Neck Pain	84.42%	65
Fatigue	46.75%	36
Headaches	35.06%	27
Twisting of the neck	76.62%	59
Muscle spasms	72.73%	56
Other (please specify)	23.38%	18
Total Respondents: 77		

Question 2: How do ongoing symptoms affect your day-to-day life? Check all that apply.

Answer Choices	Responses	
Limit you from working	53.25%	41
Limit you from participating in leisure activities and socializing	71.43%	55
Affect your quality of sleep	48.05%	37
Make you tired and listless	59.74%	46
Affect you emotionally	71.43%	55
Other (please specify)	20.78%	16
Total Respondents: 77		

Question 3: What limitations have you experienced because of having cervical dystonia? Check all that apply.

Answer Choices	Responses	
Unable to work	57.14%	40
Unable to socialize, or participate in family or leisure activities	55.71%	39
Unable to be physically active	68.57%	48
Unable to be sleep	35.71%	25
Other (please specify)	31.43%	22
Total Respondents: 70		

Question 4: Since diagnosis, which of the following therapies have you used to treat your dystonia? Check all that apply.

Answer Choices	Responses	
Neurotoxin Injection: BOTOX (onabotulinumtoxinA)	85.33%	64
Neurotoxin Injection: Xeomin (incobotulinumtoxinA)	21.33%	16
Neurotoxin Injection: Dysport (abobotulinumtoxinA)	0.00%	0
Deep Brain Stimulation (DBS)	6.67%	5
Oral medication(s)	65.33%	49
Massage therapy / Chiropractic therapy	62.67%	47
Other (please specify)	40.00%	30
Total Respondents: 75		

Question 5: Which of the following therapies are you using currently. Check all that apply.

Answer Choices	Responses	
Neurotoxin: BOTOX (onabotulinumtoxinA)	64.86%	48
Neurotoxin: Xeomin (incobotulinumtoxinA)	13.51%	10
Neurotoxin: Dysport (abobotulinumtoxinA)	0.00%	0
Oral medication(s)	45.95%	34
Massage therapy / Chiropractic therapy	33.78%	25
Other (please specify)	31.08%	23
Total Respondents: 74		

Question 6: How effective is your current therapy at controlling the following symptoms of cervical dystonia we got the following responses?

	Excellent	Very Good	Good	Just OK	Not Effective	N/A	Total	Weighted Average
Headache	6.15% 4	9.23% 6	29.23% 19	21.54% 14	6.15% 4	27.69% 18	65	3.17
Neck pain	2.74% 2	20.55% 15	34.25% 25	26.03% 19	12.33% 9	4.11% 3	73	3.26
Fatigue	1.49% 1	11.94% 8	34.33% 23	28.36% 19	17.91% 12	5.97% 4	67	3.52
Neck Twisting	4.11% 3	17.81% 13	28.77% 21	21.92% 16	17.81% 13	9.59% 7	73	3.35
Difficulty Swallowing	1.54% 1	10.77% 7	20.00% 13	18.46% 12	9.23% 6	40.00% 26	65	3.38
Emotional Distress	2.82% 2	19.72% 14	23.94% 17	26.76% 19	14.08% 10	12.68% 9	71	3.34

Question 7: Are you currently taking a neurotoxin (BOTOX, Xeomin, Dysport) to treat cervical dystonia?

Answer Choices	Responses	
Yes: Neurotoxin: BOTOX (onabotulinumtoxinA)	62.34%	48
Yes: Neurotoxin: Xeomin (incobotulinumtoxinA)	12.99%	10
Yes: Neurotoxin: Dysport (abobotulinumtoxinA)	0.00%	0
No	24.68%	19
Total		77

Question 8: Which of the following adverse effects have you experienced from the neurotoxin you use to treat cervical dystonia? Check all that apply.

Answer Choices	Responses	
Muscle weakness near injection area	39.29%	22
Bruising, bleeding, pain, swelling where the injection was given	28.57%	16
Fever, cough, sore throat, runny nose, flu symptoms	14.29%	8
Dizziness, drowsiness, tired feeling	26.79%	15
Headache, muscle stiffness, neck or back pain	39.29%	22
No adverse affects	26.79%	15
Other (please specify)	30.36%	17
Total Respondents: 56		

Question 9: Have you had any difficulty accessing therapies for cervical dystonia (i.e. financial challenges, travel limitations, supply issues)? Check all that apply.

Answer Choices	Responses	
Financial challenges	59.18%	29
Unable to travel to clinical site where drug is administered/prescribed locally (treatment centre is hard to get to)	20.41%	10
Unable to access the drug through your physician or specialist	6.12%	3
Other (please specify)	40.82%	20
Total Respondents: 49		

Appendix 2: Caregivers

Question 1: What challenges do caregivers face in caring for someone with cervical dystonia? Check all that apply.

Answer Choices	Responses
Access to appropriate therapies and/or medications	33.33% 1
Access to specialty physicians / movement disorder specialists	66.67% 2
Cost of caring for patients / cost for treatment	33.33% 1
Demands on personal time	66.67% 2
Managing work and caregiving	33.33% 1
Other (please specify)	66.67% 2
Total Respondents: 3	

Question 2: How do current treatments impact the role of the caregiver? Check all that apply.

Answer Choices	Responses
Require frequent movement disorder specialist visits	100.00% 2
Treatments cause side effects that require additional care giving	50.00% 1
Are expensive and affect income levels	50.00% 1
Income level affected by inability to work due to caregiving	50.00% 1
Other (please specify)	50.00% 1
Total Respondents: 2	

Question 3: Are there challenges for caregivers in dealing with the adverse effects related to the current therapy/therapies a loved one is taking? Check all that apply.

Answer Choices	Responses
Muscle weakness near injection area	0.00% 0
Bruising, bleeding, pain, swelling where the injection was given	0.00% 0
Fever, cough, sore throat, runny nose, flu symptoms	0.00% 0
Dizziness, drowsiness, tired feeling	0.00% 0
Headache, muscle stiffness, neck or back pain	0.00% 0
No adverse effects	0.00% 0
Other (please specify)	100.00% 2
Total Respondents: 2	