Optimizing Access to Care for Patients with Idiopathic Pulmonary Fibrosis

Backgrounder for National Forum

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IHE INSTITUTE OF HEALTH ECONOMICS ALBERTA CANADA

CANADIAN PULMONARY FIBROSIS FOUNDATION

Boehringer Ingelheim

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CANADIAN PULMONARY FIBROSIS FOUNDATION

The Canadian Pulmonary Fibrosis Foundation (CPFF) is a registered Canadian Charitable Foundation established to provide hope and support for people affected by pulmonary fibrosis. Robert Davidson, president of the CPFF, created the organization in 2009 to raise money to find causes and treatments for PF, provide education and support for people affected by pulmonary fibrosis, and help answer those non-medical questions frequently asked by those suffering with the disease. The CPFF is a not-for-profit charitable organization.

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BACKGROUND

In October 2015, we held the first Canadian consensus discussion that led to provisional recommendations regarding special considerations for policy-making and healthcare decision-making for interventions in patients with idiopathic pulmonary fibrosis (IPF). The meeting included 11 representatives of key stakeholders: patients, care providers, and policy researchers from across Canada. The purpose of the meeting was to discuss the current state of evidence and information regarding IPF. This led to draft recommendations for policymakers and healthcare administrators who must make purchasing or reimbursement decisions regarding the care and treatment of patients with IPF.

Participants first discussed factors that require consideration when implementing service for IPF patients. The following key factors were identified:

- There are many important considerations for IPF that are not specific to IPF, and apply to other degenerative, chronic, and fatal conditions (such as amyotrophic lateral sclerosis [ALS], cystic fibrosis, and oncology) that also require a focus on palliation and providing end-of-life support as well as symptom management.
- Unlike other similarly debilitating diseases, there may be less awareness of the severity of IPF as well as less available resources, compared to these other diseases.
- An important consideration is the value of integrated care approaches, especially as it is convenient for patients and can improve patient experiences through providing effective patient navigation, improving diagnostic accuracy (and reducing unnecessary utilization of services, including new interventions), improving specialist productivity, and creating a platform for standardized approaches to care.
- Given the above, any new intervention for IPF has the potential to be more effective and cost-effective in the context of a multidisciplinary team.
- Because of considerable uncertainty regarding emerging and existing treatments, it is important to consider how to collect information on an ongoing basis, in order to best revisit past decisions and re-assess available interventions.

The DRAFT recommendations were based on an examination of evidence and current international considerations for IPF and other rare diseases, and have now became the basis of the development of a "Canadian Patient Charter" and the basis of this National Forum. The DRAFT recommendations for policymakers can be found at: *http://www.ihe.ca/publications/optimizing-access-to-care-for-patients-with-idiopathic-pulmonary-fibrosis-summary-report-and-draft-recommendations*.

The recommendations, which are criteria presented as a series of questions to guide decision-making, appear in Table 1 along with a rationale for each. Further explanation of this table appears afterwards.

TABLE 1: Checklist for Policymakers

#	Heading	Question	Rationale
1.	**Consideration of clinical benefit** Health-related quality of life	How does the new treatment affect the way that patients feel and function? Is the measure to determine this effect valid?	• IPF has dramatic impact on HRQL • There are no gold standard measures
	Quality of death and dying	Does the new intervention improve the many dimensions of the experience of dying that go beyond simple control of physical distress?	• IPF is a fatal disease and requires consideration beyond HRQL
	Effect on survival and disease progression	Does the treatment likely affect survival or reliable measures of disease progression?	• IPF is a fatal disease and patients and caregivers value longer survival
2.	**Consideration of patient values**	Were patients consulted regarding their current experience with the disease and what they would value with a new treatment?	• IPF is not well understood
3.	**Consideration of severity/morbidity of the disease including premature death**	Do decisions regarding policies that affect the management and treatment of IPF consider the life-threatening nature of illness?	• Diseases that are significantly life-threatening warrant special consideration
4.	**Availability of alternatives**	Do decisions regarding policies that affect the management and treatment of IPF consider the number of available alternatives?	• There are few alternatives to effectively treat IPF • Lung transplant is the only treatment that can prolong survival
5.	**Wider consultation with stakeholders**	Were other key stakeholders, including formal and informal caregivers, consulted regarding policies that affect the management of IPF?	• IPF is a complex disease to manage • Informal caregivers may have special insights
6.	**Starting and stopping rules and rationing of service**	Is a stopping rule being considered?	• Stopping rules may have a significantly negative impact on patients • There are viable alternatives to stopping rules
7.	**Integrated care centres and dedicated idiopathic pulmonary fibrosis sub-specialists**	Are new policies that affect the management and treatment of patients considering how and where care will be delivered?	• Integrated care centres as a means of reducing inappropriate utilization through improved diagnostic accuracy, and as a means to increase the effectiveness (and cost-effectiveness) of treatment

DRAFT RECOMMENDATIONS FOR POLICYMAKERS: EXPLANATIONS AND RATIONALE

The need for recommendations in general

Idiopathic pulmonary fibrosis is a disease characterized by loss of health-related quality of life and premature mortality. There are several reasons that IPF patients may warrant special considerations for healthcare policy-making that applies to populations. Firstly is the low number of identifiable people diagnosed with illness. Although the exact number of Canadians with IPF (that is, disease prevalence) is unknown, best estimates of the number of Canadians with established disease, based on international studies, range from 5,000 to 9,000 Canadians, or an estimated 10 to 25 per 100,000 of the existing population.[1] This makes IPF a rare disease, or consistent with more common international definitions of rare disease that use a threshold between 40 and 50 cases per 100,000 people (global average was 40 per 100,000 people).

A second factor that may warrant special consideration is the severity of the disease and lack of available treatment options, factors that Canadian and international studies of social values have shown that the public values.[2] These factors are also considered by licensed product (Health Canada) and reimbursement (Common Drug Review) regulators when deciding to create special priority for reviews and ultimately access to care. The rapid loss of function and rapid mortality from IPF also makes this disease similar to many cancers, requiring intensive treatment and palliative therapy with considerations of end-of-life care. Current data suggests that 50% of patients die, usually from lung failure, between two and five years from the time of diagnosis, with younger patients typically surviving longer.[3]

Rationale for specific recommendations

When deciding on whether to fund new goods and services (that is, innovative products, processes, or approaches to care) for patients with IPF, the panel recommends that healthcare policymakers consider using criteria.

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[1] IPF Fact Sheet based on data on file from InterMune Canada, Inc.

[2] Nick Dragojlovic et al., "Challenges in measuring the societal value of orphan drugs: Insights from a Canadian stated preference survey," *The Patient* 8, no. 1 (2015): 93-101, doi:10.1007/s40271-014-0109-5; Warren G. Linley and Dyfrig A. Hughes, "Societal views on NICE, cancer drugs fund and value-based pricing criteria for prioritising medicines: A cross-sectional survey of 4118 adults in Great Britain," *Health Economics* 22, no. 8 (August 2013): 948-64, doi:10.1002/hec.2872; Emmanouil Mentzakis, Patricia Stefanowska, and Jeremiah Hurley, "A discrete choice experiment investigating preferences for funding drugs used to treat orphan diseases: An exploratory study," *Health Economics, Policy, and Law* 6, no. 3 (July 2011): 405-33, doi:10.1017/S1744133110000344; Arna S. Desser, Jan Abel Olsen, and Sverre Grepperud, "Eliciting preferences for prioritizing treatment of rare diseases: The role of opportunity costs and framing effects," *PharmacoEconomics* 31, no. 11 (November 2013): 1051-61, doi:10.1007/s40273-013-0093-y; Arna S. Desser et al., "Societal views on orphan drugs: Cross sectional survey of Norwegians aged 40 to 67," *BMJ* (Clinical Research Ed.) 341 (2010): c4715.

[3] Brett Ley and Harold R Collard, "Epidemiology of idiopathic pulmonary fibrosis," *Clinical Epidemiology* 5 (November 25, 2013): 483-92, doi:10.2147/CLEP.S54815; American Thoracic Society and others, "Idiopathic pulmonary fibrosis: Diagnosis and treatment. International Consensus Statement," 2000, *http://dspace.iss.it/srdspace/handle/2198/930*.

Recommendation 1: Consideration of clinical benefit

- Health-related quality of life
  - How does the new treatment affect the way that patients feel and function?
  - Is the measure to determine this effect valid?
- Quality of death and dying
  - Does the new intervention improve the many dimensions of the experience of dying that go beyond simple control of physical distress?
- Effect on survival and disease progression
  - Does the treatment likely affect survival or reliable measures of disease progression?

Rationale: Studies that have explored what experiences and outcomes are important to patients have identified several emerging themes regarding how IPF negatively impacts quality of life. This includes frustration with diagnosis and management of care, a lack of information about their disease, negative perception from decreased libido or inability to continue sexual activity, reduced independence and the need to rely on friends and family, difficulties with carrying on relationships, and financial concerns with a diminished ability to work.[4]

Patient input on new drug applications to the Canadian Agency for Drugs and Technologies in Health (CADTH), gathered by the Canadian Pulmonary Fibrosis Foundation, similarly indicates these concerns. Patients have also acknowledged the limitations of existing treatments and the need for a treatment that will meaningfully slow the progress of disease in the absence of a cure.[5] All respondents to a survey of 217 Canadian IPF patients and caregivers indicated they hoped to slow the progression of the disease to allow them greater quality of life. This is also consistent with other formal studies in the area, which also indicate patient enthusiasm for trying new therapies, especially those that might change disease course.[6]

To capture how patients with IPF feel and function during the disease course, generic instruments that capture health-related quality of life (HRQL), such as the Short-Form-36 (SF-36) survey and Saint George's Respiratory Questionnaire (SGRQ), have been used and shown to be sensitive to changes in disease progression.[7] However, it has been increasingly recognized that these instruments may not be suitable for capturing all relevant information (that is, either quality of life "domains" or information that informs these) or may capture information that is not important to patients. Other disease-specific measures have been or are being developed but may require further validation; currently, there is no gold standard instrument for measuring impact on HRQL.

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[4] Jeffrey J. Swigris et al., "Patients' perspectives on how idiopathic pulmonary fibrosis affects the quality of their lives," *Health and Quality of Life Outcomes* 3 (2005): 61, doi:10.1186/1477-7525-3-61.

[5] CADTH, Common Drug Review CDEC Final Recommendation - Pirfenidone resubmission. Notice of final recommendation, April 15, 2015.

[6] Amanda Belkin and Jeffrey J. Swigris, "Patient expectations and experiences in idiopathic pulmonary fibrosis: Implications of patient surveys for improved care," *Expert Review of Respiratory Medicine* 8, no. 2 (April 2014): 173-78, doi:10.1586/17476348.2014.880056.

[7] J. A. Chang et al., "Assessment of health-related quality of life in patients with interstitial lung disease," *Chest* 116, no. 5 (November 1999): 1175-82.

Given significant "knowledge gaps" associated with existing patient-reported outcome measures (such as the SGRQ),[8] there have been some attempts to develop IPF-specific measures that better capture relevant experience. While the SGRQ has been demonstrated to be "useful",[9] an SGRQ instrument modified to more directly measure experiences in IPF patients has been developed.[10] Another tool, A Tool to Assess Quality of Life in Idiopathic Pulmonary Fibrosis (ATAQ-IPF), has also been developed,[11] and validation across countries has been performed.[12]

Recommendation 2: Consideration of patient values

- Were patients consulted regarding their current experience with the disease and what they would value with a new treatment?

Rationale: The importance of patient and citizen involvement in health care and decision-making has grown in prominence. It is also well identified and promoted in a number of WHO reports, including the Ottawa Charter[13] and disease-specific issues on malaria[14] and tuberculosis.[15] Participants in the previous IHE consensus conference on IPF noted that, unlike other similarly debilitating diseases, there may be less awareness of the severity of IPF as well as less available resources, compared to these other diseases.

Recommendation 3: Consideration of severity/morbidity of the disease including premature death

- Do decisions regarding policies that affect the management and treatment of IPF consider the life-threatening nature of illness?

Rationale: IPF is a fatal condition with no cure (other than lung transplantation) or treatments to stop disease progression. The disease course is rapid with distressing symptoms of dyspnea, and 50% of patients die within four years of diagnosis. There is considerable evidence to suggest society places an increased value on improvements in health for relatively fatal illnesses.

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[8] Jeffrey J. Swigris and Diane Fairclough, "Patient-reported outcomes in idiopathic pulmonary fibrosis research," *Chest* 142, no. 2 (August 2012): 291-97, doi:10.1378/chest.11-2602.

[9] Jeffrey J. Swigris et al., "The psychometric properties of the St George's Respiratory Questionnaire (SGRQ) in patients with idiopathic pulmonary fibrosis: A literature review," *Health and Quality of Life Outcomes* 12 (2014): 124, doi:10.1186/s12955-014-0124-1.

[10] Janelle Yorke, Paul W. Jones, and Jeffrey J. Swigris, "Development and validity testing of an IPF-specific version of the St George's Respiratory Questionnaire," *Thorax* 65, no. 10 (October 2010): 921-26, doi:10.1136/thx.2010.139121.

[11] Jeffrey J. Swigris et al., "Development of the ATAQ-IPF: A tool to assess quality of life in IPF," *Health and Quality of Life Outcomes* 8 (2010): 77, doi:10.1186/1477-7525-8-77.

[12] Janelle Yorke et al., "Cross-Atlantic modification and validation of the A Tool to Assess Quality of Life in Idiopathic Pulmonary Fibrosis (ATAQ-IPF-cA)," *BMJ Open Respiratory Research* 1, no. 1 (2014): e000024, doi:10.1136/bmjresp-2014-000024.

[13] World Health Organization, *Ottawa Charter for Health Promotion* (Geneva, Switzerland: World Health Organization, 1986).

[14] World Health Organization, *Community Involvement in Rolling Back Malaria* (Geneva, Switzerland: World Health Organization, 2002).

[15] World Health Organization, *The ENGAGE-TB Approach: Operational Guidance Integrating Community-Based Tuberculosis Activities into the Work of Nongovernmental and Other Civil Society Organization* (Geneva, Switzerland: World Health Organization, 2012).

Recommendation 4: Availability of alternatives

- Do decisions regarding policies that affect the management and treatment of IPF consider the number of available alternatives?

Rationale: There are no available alternatives shown to prolong survival, other than lung transplantation. Some pharmacological therapies have been shown to alter disease course. Lung transplantation remains the single evidence-based option for prolonging survival in patients with IPF. However, there are no formal evaluations of its cost-effectiveness. There are similarly no economic evaluations of other non-drug approaches to care, including how care is delivered and organized (that is, through specialty clinics), or the use of disease management programs, education, and other supportive measures.

Recommendation 5: Wider consultation with stakeholders

- Were other key stakeholders, including formal and informal caregivers, consulted regarding policies that affect the management of IPF?

Rationale: Due to the complexity of the disease and evolving information regarding its treatment, dedicated sub-specialists and other care providers should be consulted. Participants in the previous IHE consensus meeting highlighted that the complex nature of the disease means consulting with experts (and patients) is required in order to avoid misapplying thinking from other diseases that appear to be (but are not) similar, such as COPD.

Recommendation 6: Starting and stopping rules and rationing of service

- Is a stopping rule being considered?

Rationale: Due to the effect on patients, alternatives to stopping rules should be considered unless there is clear and compelling evidence to support them. Policymakers should consider outcomes-based risk sharing arrangements as one potential alternative, which can be implemented through linking jurisdictional administrative data to an existing national registry (the Canadian Registry for Pulmonary Fibrosis [CARE-PF]). This will provide an opportunity to revisit decisions. Other alternatives may include limiting new treatments to narrow subpopulations who will receive the greatest societal benefit, or entering financial risk sharing agreements that account for increased expenditure (and potential benefit) when a stopping rule is not applied.

Recommendation 7: Integrated care centres and dedicated idiopathic pulmonary fibrosis sub-specialists

- Are new policies that affect the management and treatment of patients considering how and where care will be delivered?

Rationale: New treatments should be restricted to dedicated sub-specialists or integrated care centres as a means of reducing inappropriate utilization through improved diagnostic accuracy, and as a means to increase the effectiveness (and cost-effectiveness) of treatment. Opportunities to fund these centres should be considered when negotiating prices for highly expensive treatments.