Australian Government

Medical Services Advisory Committee

Public Summary Document

Application No. 1510.1 – Emicizumab for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with haemophilia A with factor VIII inhibitors

Applicant: **Roche Products Pty Ltd**

Date of MSAC consideration: **MSAC 76th Meeting, 1-2 August 2019**

Context for decision: MSAC makes its advice in accordance with its Terms of Reference, visit the MSAC website

1. Purpose of application

A resubmission providing a proposal for weighted pricing, including an annual price reduction for use across all requested patients with haemophilia A (HMA) was received from Roche Products Pty Ltd by the Department of Health.

2. MSAC's advice to the Minister

After considering the strength of the available evidence in relation to comparative safety, clinical effectiveness and cost-effectiveness, MSAC supported funding of emicizumab via the National Blood Authority on the basis of acceptable clinical effectiveness and safety compared with by-passing agents (BPAs) in patients with haemophilia A with factor VIII inhibitors. MSAC advised on the appropriate prices for different subpopulations it considered should be applied (in setting the overall subsidy price), and in the calculation of the financial impact of funding. MSAC considered the applicant's proposed risk sharing arrangement (RSA) was not adequate for addressing risk of use outside the proposed patient population.

Specifically, MSAC advised that this support was subject to pricing negotiations, based on the following shadow prices in the re-calculation of weighted average price and the proposed RSA, to be implemented alongside the related shadow prices in the MSAC advice for Application 1579 (HMA without FVIII inhibitors), noting some of the shadow prices relate to possible use of emicizumab beyond that requested:

- patients with HMA and inhibitors who previously received BPA prophylaxis: $**redacted**/mg
- patients with HMA and inhibitors who previously received BPA on demand: $**redacted**/mg
- patients with HMA and inhibitors who previously received interrupted immune tolerance induction therapy: $**redacted**/mg.

Consumer summary

Roche Products Pty Ltd applied for public funding for routine administration of emicizumab to prevent or reduce the frequency of bleeding episodes in patients with haemophilia A with factor VIII inhibitors.

People with haemophilia A (HMA) cannot clot blood properly, which results in spontaneous bleeds or excessive bleeding from injury. People with severe or moderate HMA are often treated with the clotting factor VIII. However, some people develop inhibitors to FVIII, which means FVIII no longer works. They mostly receive another kind of medicine to prevent or stop bleeds, called bypassing agents (BPAs).

This application is for emicizumab, a medicine that also reduces rates of bleeding and also reduces the need for BPAs to manage those bleeds which do occur. People who have inhibitors to FVIII can use emicizumab effectively.

MSAC's recommendation to the Commonwealth Health Minister

MSAC considered that emicizumab is easier to administer and works better than BPAs for people who have FVIII inhibitors, and is acceptably safe. MSAC supported public funding on the National Product List for people with HMA who have FVIII inhibitors. However, MSAC advised that the application did not justify the expense of emicizumab for this subpopulation, and this supportive advice was subject to pricing negotiations with the applicant.

3. Summary of consideration and rationale for MSAC's advice

MSAC recalled its consideration of Application 1510 at its November 2018 meeting, where it accepted the clinical need for emicizumab, and that, compared with BPAs, emicizumab decreases bleed frequency, is easier to administer and has an acceptable safety profile. However, MSAC had queried the cost-effectiveness of emicizumab in patients with FVIII inhibitors, as it was compared with BPAs, which have a high cost per patient and had not been assessed for cost-effectiveness. MSAC noted the resulting high cost of emicizumab per patient, and considered that this cost would need to decrease before it could consider emicizumab to be acceptably cost-effective for the HMA population with FVIII inhibitors. MSAC also expressed concern about leakage into the HMA population without inhibitors.

This resubmission proposed a weighted average price arrangement across the requested HMA subpopulations with and without FVIII inhibitors and an associated risk sharing arrangement (RSA). MSAC noted that these prices are contingent on funding emicizumab for both populations. The applicant proposed a shadow price of $**redacted**/mg for all requested patients without inhibitors and $**redacted**/mg for all requested patients with inhibitors in year 1 (estimating a weighted average price of $**redacted**/mg). The shadow price for the population with inhibitors would then decrease each year to $**redacted**/mg at year 5, with the shadow price for the population without inhibitors remaining the same, giving a weighted average price of $**redacted**/mg in year 5. These proposed shadow prices were contingent on funding emicizumab for both populations before the end of 2019. For the reasons given in its November 2018 consideration, MSAC did not accept these shadow prices as a basis to support public funding for patients with HMA and inhibitors.

The resubmission then calculated weighted average prices reflecting the applicant's estimated ratio of 9% with inhibitors to 91% without inhibitors in the target HMA population. MSAC noted that the population with inhibitors would likely decrease over time, but so slowly as

unlikely to affect the 4-year timeframe of the proposed initial RSA. MSAC also noted that Australian Bleeding Disorders Registry (ABDR) data shows that the proportion of patients with inhibitors is approximately 2% in the overall HMA population, not 9%. MSAC considered that this was a significant difference in estimate and the true proportion needs to be determined.

MSAC noted the resubmission also proposed expenditure caps for emicizumab of $**redacted** million in year 1, increasing each year to $**redacted** million in year 5. If an expenditure cap is exceeded, the lower price of $**redacted**/mg would apply to all emicizumab usage beyond the cap. MSAC considered this RSA to be inadequate, as it would not disincentivise treating mild patients and thus does not address the concern of leakage beyond the requested subpopulations. MSAC noted that, currently, very few patients with mild HMA are treated prophylactically, but the RSA should still account for emicizumab prophylaxis as a future possibility for such patients. MSAC also noted that the RSA is based on the cost of FVIII. MSAC considered that the cost of FVIII would decrease in the future, and thus the cost of emicizumab could be linked to the falling cost of FVIII.

MSAC advised that using the proposed weighted average price was not adequately justified in economic terms, and would also still result in uncertainties for overall financial impact, as on-demand patients are not accounted for, and leakage into the mild and untreated HMA populations remains a concern. On-demand patients have a higher annualised bleeding rate as compared to those receiving prophylaxis, and treating these bleeds is costly and must be factored into the financial calculations. In addition, the applicant's RSA did not eliminate all financial risks of leakage.

In this context, MSAC also noted the strong consumer and industry support for emicizumab, and their expectations of an increase in patient quality of life and flow-on consequences for families and productivity associated with using it compared with BPAs.

MSAC therefore advised that alternative approaches were needed to generate shadow prices and a revised RSA for emicizumab across all the affected HMA subpopulations, whether requested or not. These approaches are outlined in more detail in the attachment. MSAC further advised that the most recent ABDR data be used in calculating these shadow prices and so the calculations and results of this approach in this MSAC-ratified document were finalised using ABDR data that became available soon after the MSAC meeting itself.

As part of its advice for Application No. 1579 (HMA without FVIII inhibitors), MSAC advised that the application's economic evaluation provided an acceptable basis for calculating a price for emicizumab of $**redacted**/mg for patients with severe HMA without inhibitors who previously received FVIII prophylaxis.

For patients starting emicizumab who previously received prophylaxis, MSAC advised that the shadow price for emicizumab be linked to its ability to reduce annualised bleed rates, noting the consequences of bleeds for patient outcomes and extra costs. MSAC noted the growing clinical trend towards achieving zero annualised bleeds, and accepted this as clinically valuable, and possibly more important clinically. However, MSAC considered that estimates of annualised bleed rates have been estimated with greater confidence, and are therefore a preferred basis by which to benchmark shadow prices for emicizumab for patients starting emicizumab who previously received prophylaxis.

At the requested price of $**redacted**/mg justified by the applicant using a cost- consequences analysis for patients with severe and moderate HMA without inhibitors who previously

received FVIII prophylaxis, MSAC calculated the cost per annualised bleed for severe HMA patients on a cost-minimisation basis as $**redacted**/(4.8-1.5) = $**redacted**/mg

Applying this cost per annualised bleed averted for patients with inhibitors who previously received BPA prophylaxis, and reflecting 2.9 bleeds per year for emicizumab from Arm A of HAVEN 1 and 15.7 bleeds per year for BPAs from the NIS BPA study, gives: (15.7-2.9) x $**redacted** = $**redacted**/mg.

MSAC advised that, to generate a corresponding shadow price for emicizumab in patients with inhibitors who previously received a BPA on demand, a similar approach should be taken as advised for patients without inhibitors, which gives: $**redacted** x (($**redacted**) = $**redacted**/mg.

MSAC noted that the ABDR data indicated that some patients with inhibitors were receiving immune tolerance induction therapy. MSAC considered it highly unlikely that such a course of therapy would be interrupted to start emicizumab, so advised that the shadow price of $**redacted**/mg could be used for this unlikely use beyond the requested subpopulations.

MSAC further advised that the Department of Health should negotiate with the applicant to finalise how these shadow prices should be aggregated to generate the weighted average price for inclusion in the financial analyses for all requested subpopulations (including patients without inhibitors), and also how they should be best incorporated into the proposed RSA to account for any usage of emicizumab in patients with HMA beyond the requested subpopulations. Noting the proposed 4-year timeframe of this proposed initial RSA, MSAC also advised that the fact of its existence should be public, and it should not inadvertently exclude the possibility of funding other emerging therapies for HMA.

Reflecting the residual uncertainty about expected rates of emicizumab uptake across the subpopulations, MSAC advised that this approach would also inform a review 12 to 24 months after emicizumab listing using data from the ABDR and haemophilia treatment centres to confirm the nature of the previous treatment and the HMA severity for each patient who starts emicizumab. Further, as this approach is most relevant to the prevalent pool of patients at the time that emicizumab is listed, consideration of how this approach might need to deal with incident patients could be considered at the time of this recommended review.

For implementation purposes, MSAC advised that prescription of funded emicizumab should be limited to haematologists working in Haemophilia Treatment Centres, and that BPAs would still be needed to manage breakthrough bleeds.

MSAC noted that cost-effectiveness studies have never been used to inform the existing prices for BPAs, making economic evaluations difficult for applications such as this which rely on accepting that BPAs are acceptably cost-effective. MSAC wondered if it would be informative to commission a study to identify the reduction in prices at which BPAs would be acceptably cost-effective, especially since there are products on the horizon that are similar to emicizumab, and that would likely be put forward for public funding in the future. Such treatments are currently in Phase 2 trials.

4. Background

At its November 2018 meeting, MSAC did not support emicizumab for routine prophylaxis to prevent bleeding or reduce frequency of bleeding episodes in patients with haemophilia A with factor VIII inhibitors. MSAC accepted that there was strong evidence that emicizumab substantially reduced the frequency of bleeding episodes; however, MSAC was concerned

that the economic justification for funding came from reductions in current practice, which has not itself been shown to be acceptably cost-effective. Rather than consolidate potentially cost-ineffective practice, MSAC requested that the cost-effectiveness of current practice be determined as a means to then determine the cost-effectiveness of emicizumab in the proposed population. MSAC was also concerned that there were practical difficulties in ensuring that, over time, emicizumab would remain limited to the proposed target population [Public Summary Document (PSD) Application No. 1510.1 2018, p1].

The Department outlined two options for consideration in order to progress Application 1510. The first option was for the applicant to undertake the review of current practice involving bypass agents (BPAs) as outlined in the 1510 PSD, noting that this would be an extended and uncertain process requiring considerable resources. The second option was to adopt the reduced price offered in Application 1579 across all requested patients. The applicant instead offered a weighted pricing approach across all requested HMA patients, involving **redacted**%/year reductions over the first five years of listing.

5. Prerequisites to implementation of any funding advice

Refer to Application 1510 PSD 2018, p3 for details of the four relevant items listed on the ARTG.

6. Proposal for public funding

The prices offered to the NBA for the inhibitor subgroup (Application 1510), considered by MSAC at its November 2018 meeting are show in Table 1. These prices are equivalent to $**redacted**/mg.

Table 1 Previous price for emicizumab in inhibitor patients

Product Type	Name	Presentation	Supplier	Price
Emicizumab	HEMLIBRA®	30 mg/1 mL (30 mg/mL), 1 vial 60 mg/0.4 mL (150 mg/mL), 1 vial 105 mg/0.7 mL (150 mg/mL), 1 vial 150 mg/1 mL (150 mg/mL), 1 vial	Roche Products Pty Ltd	$**redacted** $**redacted** $**redacted** $**redacted**

The new varying shadow prices of emicizumab proposed for the inhibitor subgroup in this resubmission remains $**redacted**/mg in Year 1, then reducing gradually each year to $**redacted**/mg (representing a maximum **redacted**% price reduction) in Year 5.

The new weighted prices of emicizumab proposed across the inhibitor and non-inhibitor subgroups in this resubmission (Table 2) are equivalent to $**redacted**/mg in Year 1, then reducing by **redacted**% each year to $**redacted**/mg in Year 5 (these weighted prices also reflect a fixed shadow price of $**redacted**/mg in the larger non-inhibitor subgroup).

Table 2 Proposed prices of emicizumab

Strength	30 mg	60 mg	105 mg	150 mg
Proposed price MSAC Application 1510 (INH) and Application 1579 (NIH)				
Original INH price	$redacted	$redacted	$redacted	$redacted
Original NIH price	$redacted	$redacted	$redacted	$redacted
Proposed weighted INH + NIH price: Years 1-5				
Year 1	$redacted	$redacted	$redacted	$redacted
Year 2	$redacted	$redacted	$redacted	$redacted
Year 3	$redacted	$redacted	$redacted	$redacted
Year 4	$redacted	$redacted	$redacted	$redacted
Year 5	$redacted	$redacted	$redacted	$redacted

INH = haemophilia A with FVIII inhibitors; NIH = haemophilia A without FVIII inhibitors

Source: Emicizumab Integrated INH+NIH BIA.xlsx, worksheet 'Vial combinations INH' and 'Vial combinations NIH'

The resubmission stated that the weighted price across the two subgroups was calculated using an epidemiological approach, which assumes uptake in 9% inhibitor and 91% non-inhibitor patients across the total emicizumab-treated subgroup; for example, in Year 1:

($**redacted** x 9%) + ($**redacted** x 91%) = $**redacted**.

The proposed price reduction of **redacted**% per year for the weighted prices provide greater proportional reductions for the shadow prices for the inhibitor subgroup.

7. Summary of Public Consultation Feedback/Consumer Issues

During the resubmission phase (application 1510.1), one response was received from a professional organisation, expressing disappointment with MSAC's recommendation for application 1510, and asking that the recommendation be reconsidered (through resubmitted application 1510.1). The feedback highlighted the life-changing benefits of subcutaneous and less frequent injections, with evidence demonstrating reduced bleeding episodes in patients with inhibitors. The feedback also claimed that current intravenous bypassing agents are predominantly used on-demand, after bleeding episodes that are costly, with worsening morbidity and increased mortality risk for patients.

8. Proposed intervention's place in clinical management

This was unchanged, refer to Application 1510 PSD 2018, p4.

9. Comparator

This was unchanged, refer to Application 1510 PSD 2018, p5.

10. Comparative safety

This was unchanged, refer to Application 1510 PSD 2018, p5.

11. Comparative effectiveness

This was unchanged, refer to Application 1510 PSD 2018, pp6-7.

12. Economic evaluation

The applicant did not provide new estimates for the economic evaluation, refer to Application 1510 PSD 2018, pp7-9.

13. Financial/budgetary impacts

Consistent with the previous application, an epidemiological approach was used to estimate the financial implications (Table 3), which included an assumption that freed hospital resources would be realised as financial savings, rather than being redeployed. ESC noted that, as the shadow price per mg for emicizumab for the population with inhibitors in Year 1 is unchanged from Application 1510, the total price per patient per year is the same as that considered by MSAC at its November 2018 meeting. This shadow price reduces by roughly the same proportion each year to a maximum **redacted**% reduction from Year 5.

Table 3 Total net cost of listing emicizumab

	Year 1	Year 2	Year 3	Year 4	Year 5
Patients treated					
Total patients treated - INH	19	34	35	35	36
Total patients treated - NIH	158	366	398	405	413
Total patients treated - INH + NIH	**178**	**400**	**433**	**441**	**449**
Net cost to government – INH subgroup					
Cost of emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Other costs associated with emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted treatments	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted hospitalisation	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Net cost to government – INH	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Net cost to government – NIH subgroup					
Cost of emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Other costs associated with emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted treatments	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted hospitalisation	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Net cost to government - NIH	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Overall net cost to government – INH + NIH					
Cost of emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Other costs associated with emicizumab	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted treatments	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Cost of substituted hospitalisation	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**
Overall net cost to government - INH + NIH	$**redacted**	$**redacted**	$**redacted**	$**redacted**	$**redacted**

INH = haemophilia A with FVIII inhibitors; NIH = haemophilia A without FVIII inhibitors

Source: Integrated Section E: Emicizumab Integrated INH+NIH BIA.xlsx, worksheet 'Overall net cost to gov INH+NIH'

14. Key issues from ESC for MSAC

ESC key issue	ESC advice to MSAC
Weighting of price may not accurately reflect the relative proportion of patients with and without inhibitors, and changes in this relative proportion over time once emicizumab is available	This changes the overall spend. Implementation of a risk share arrangement (RSA), including with early review within 1–2 years of listing, using utilisation data from the NBA and possibly supplemented with switch data from the Australian Bleeding Disorders Registry (ABDR), could help mitigate the risk of unjustified expenditure.
Emicizumab used beyond HMA patients with a need for routine factor VIII (FVIII) prophylaxis (emicizumab price has been cost-minimised to FVIII prophylaxis not FVIII on-demand)	This changes both the cost-effectiveness and overall spend. Ensure total number of patients treated for the purpose of the proposed risk share arrangement is not greater than the current use of FVIII in the requested subgroup(s) as per ABDR data. Consider what price of emicizumab is justified for any use beyond this amount.
Emicizumab used beyond patients with moderate or severe HMA (including emicizumab used by patients with mild HMA	This changes both the cost-effectiveness and overall spend. Ensure total number of patients treated for the purpose of the proposed risk share arrangement is not greater than the current use of FVIII in the requested subgroup(s) as per ABDR data. Consider what price of emicizumab is justified for any use beyond this amount.
Whether the reduction in price offered for the subgroup with FVIII inhibitors satisfactorily addresses MSAC's concerns regarding the previously high annual cost per patient for emicizumab, and the absence of any formal cost-effectiveness analysis for bypass agents (BPAs)	It is difficult for ESC to comment on this. From a technical perspective, it would have been informative to understand the cost-effectiveness of BPAs, without which there is no new basis to reconsider the shadow prices offered for this subgroup. The resubmission instead emphasises the weighted prices across the two requested subgroups.
In the absence of a formal evaluation, what advice can ESC provide to facilitate MSAC's consideration of the overall proposal for emicizumab (e.g. is there a basis for concurrent consideration by MSAC of 1579 and expedited 1510.1)?	ESC notes that the two subgroups for whom listing is sought are dynamically interrelated and that over time the availability of emicizumab will likely increase the relative proportion of HMA patients without FVIII inhibitors. Consequently, the interplay between the subgroups and the impact of emicizumab treatment has implications for managing utilisation and for determining the weighted price of emicizumab. Implementing restrictions on NBA-funded use (both to specify the eligible subgroups and to limit prescribing of NPL-funded emicizumab to haematologists managing HMA), and backing this up by a risk-share arrangement are likely to be needed to ensure the intention of the requested emicizumab listing.

ESC discussion

Application 1510.1 is for National Product Listing of emicizumab for use in patients with moderate to severe haemophilia A (HMA; also called congenital factor VIII deficiency) with factor VIII (FVIII) inhibitors. The major difference between this and the original application is the price, which is achieved by seeking concurrent consideration of the HMA populations with and without inhibitors. This resubmission proposes a weighted price that is predicated on MSAC supporting the related application for patients without FVIII inhibitors (see Application 1579).

ESC noted that as a result of an agreed shortened timeframe for the resubmission there was insufficient time to complete a full independent critique of the resubmission. Rather, the Department has provided a brief overview, to which the applicant has responded. ESC considered that this was not an optimal basis on which to advise on options for listing.

ESC noted that the applicant's claim that the new weighted prices offered for the HMA population with inhibitors was a significant **redacted**% to **redacted**% reduction compared to application 1510. ESC noted that that basis of this claim was a reliance on calculating weighted prices across both HMA subgroups (i.e. with and without inhibitors), in which the shadowed price reductions in the calculations for the HMA population with inhibitors were relatively small (**redacted**% to **redacted**%).

ESC noted that patients can develop and lose inhibitors, so patients can be in flux between the two subgroups throughout their life. This makes it difficult to define the numbers of patients in each subgroup, as this may change at any given time. However, ESC noted that wider use of emicizumab may eliminate the subgroup of patients with FVIII inhibitors in the future, leaving more of the treated population in the without inhibitors subgroup (Application 1579). ESC queried whether the discounted weighted price offered for the first five years of listing was an accurate reflection of the shift of patient numbers towards the 'without inhibitors' subgroup.

ESC noted that the utilisation estimates for the with- and without-inhibitor subgroups are not well defined, in particular, wastage for the with inhibitor subgroup. ESC also noted that, compared with the 1579 application, the resubmission changed the uptake proportions in the utilisation estimates for the without inhibitor subgroup, which the applicant contends represented a more realistic combined scenario, where uptake will be higher in the initial years as people switch to emicizumab from other treatments. ESC noted that, although emicizumab uptake rates will be fluid, close monitoring of utilisation data by the NBA might help manage the risk of greater than expected use in both requested subgroups. Noting that emicizumab should not be used in combination with either FVIII prophylaxis or BPA prophylaxis (although both FVIII and BPA would need to be retained on the NPL for on-demand use in the event of a bleeding episode and for patients with mild HMA), the total numbers of patients on emicizumab should not be greater than the current numbers of patients with moderate or severe HMA receiving FVIII treatment.

ESC considered that it is of concern that the utilisation estimates have not been independently verified, and that this overarching resubmission is hampered by the lack of a usual critique. ESC queried whether the RSA should employ different weights each year to calculate the weighted prices to better reflect the relative proportion of HMA patients with and without inhibitors.

ESC noted that the leakage of emicizumab into the mild HMA subgroup is a significant risk that needs to be mitigated through a strict risk-sharing arrangement (RSA). The applicant stated that such leakage would be addressed by reinforcing the clinical restriction, weighted pricing and the RSA.

ESC commented that the appropriateness of the weighted splits is unknown, and there are unclear assumptions around costs per patient, especially as a result of reduced bleeding rates, which the applicant has not justified. Implementation of an RSA, including with early review within 1–2 years of listing, using utilisation and HMA severity data from the NBA and possibly supplemented with switch and HMA severity data from the Australian Bleeding Disorders Registry (ABDR), could help mitigate this risk.

ESC considered that any set of annual expenditure caps must be set carefully, as the manufacturer should not be reimbursed for expenditure beyond each cap.

ESC determined that it is unable to advise any further regarding the price calculations and utilisation numbers due to the complexity and inter-play of the many assumptions and calculations presented in the resubmission. ESC considered that the RSA proposed by the applicant should be more completely assessed and independently verified. ESC suggested that a more complex pricing model based on appropriate sources including the Australian Bleeding Disorders Registry data could be considered.

15. Other significant factors

Nil.

16. Applicant's comments on MSAC's Public Summary Document

While Roche welcomes MSAC's support for funding of emicizumab, Roche contends the manner in which the alternative pricing methodology as applied is inappropriate and contains multiple inaccuracies and erroneous assumptions. Roche has worked for several years with the haemophilia community to validate the assumptions and inputs used in our pricing approach. Roche will continue to work with the Department to enable access to emicizumab as a matter of priority and is confident that agreement on fair pricing can be reached. This will ensure that all eligible Australian patients are able to realise the significant benefits associated with emicizumab treatment, rather than continuing suboptimal treatment with the considerably more expensive and less effective BPAs, and that the government does not forego the substantial savings offered by emicizumab.

17. Further information on MSAC

MSAC Terms of Reference and other information are available on the MSAC Website: visit the MSAC website