Attn[redacted],NICE.

I am responding to the announcement made by NICE on Eculizumab for a—HUS patients.

On the 11th May 2011 my daughter[redacted] was admitted to The Queen Alexandra Hospital, Portsmouth. She had acute renal failure and an Hb level of 4. She was the diagnosed with HUS and subsequently a-HUS.

She was given plasma exchange and kidney dialysis to keep her alive and in the hope she would stop haemolysing . She also had immediate kidney dialysis. Both of these treatments needing a large amount of donated blood.

[redacted] then endured plasma exchange the 2nd day, then 3rd day but no sign of any improvement and although her kidney function was now zero still the HUS remained active. After another 7 days of plasma exchange and kidney dialysis there was still no improvement and so [redacted] was offered the last place on a clinical trial for Eculizumab at Devon & Exeter Hospital.

Once all the necessary paperwork was completed [redacted] agreed to try the Eculizumab as daily plasma exchange and kidney dialysis with no sign of any improvement of the disease going into remission had taken its toll and it looked like [redacted] would be spending a very long time in hospital at great expense to the NHS and what was now becoming a problem with the hospital's blood bank.

So the first dose of Eculizumab was given and the effect was immediate and life changing. The disease was halted immediately, haemolysing ceased and [redacted] was on the road to recovery. Within two weeks she was well enough to be released from hospital and return home.

[redacted] thanks to Eculizumab now works full time paying her NI and taxes and contributing to society. She is coping well with the disease and no further ill effects have been encountered. Also due to the Eculizumab being so completely effective [redacted] kidney function has returned to an almost normal level with no need for dialysis or any other expensive treatments.

[redacted] Eculizumab has been provided for her by Alexion as part of the extended trial but they have only agreed to provide until NICE publish their final decision regarding the funding of Eculizumab. So [redacted] is now in the horrible position of not knowing whether or not she will have access to the drug once the decision is final. The thought of giving up work and returning to hospital for constant invasive procedures is a huge and real fear for her.

Please be in no doubt that with Eculizamab [redacted] can live a full and giving life, without it she will be able to contribute nothing.

Yours faithfully[redacted]

[redacted]

21 March 2014

Dea

I am writing to express my concern at the recent announcement by NICE that they will not be recommending the use of eculizumab for the treatment of aHUS.

My daughter received a kidney transplant in December last year. The operation went well, and she has made a good recovery. She is returning to work next week, and hopes to be able to work full time at last in the near future. This is only possible because she has been given access to eculizumab by the CPAG decision. Her previous transplant in 1999 failed within days when the aHUS recurred.

It had been a long and difficult wait for we watched and waited for her health to deteriorate as she relied on dialysis to keep her alive. Throughout those years she remained stoic and managed to complete her education, and make a career for herself. However she was only able to work part-time because dialysis was taking its toll on her health and energy levels. Now, finally, thanks to eculizumab, she has a working kidney. She is free, at last, from food and fluid restrictions; and from dialysis. She is free to fulfil her potential, to travel, to lead the life that was so cruelly taken away from her when she became ill with aHUS in 1996. All of these things are priceless. It is inconceivable that NICE could deny others the opportunity to be helped like

The wait for a treatment for aHUS patients has already been far too long; enough is enough. It is time for NICE to make eculizumab available for all aHUS patients for as long as they need it.

Yours sincerely

Dear [redacted]

I an writing in response to the announcement made by NICE on Eculizamab for aHUS patients.

Two years ago my 6 year old grandaughter [redacted] suddenly became extremely ill and was admitted to Manchester Royal Childrens Hospital.

Her kidneys were failing and her blood counts were at rock bottom causing her to become covered in bruises. We were unsure if she would survive. She was given blood and platelet transfusions but this did not give much improvement in her condition.

The diagnosis was made of aHUS and we were told of the possibility of permenant kidney failure, possible brain and heart damage due to clots forming in her blood. All we could do was wait and pray for her.

[redacted] was offered the drug Eculizamab and we had no hesitation in agreeing to this, knowing that if she did survive she could face a very uncertain future and perhaps a lifetime on dialysis.

Within 2 days of receiving Eculizamab the improvement in her condition was amazing and her kidney function and blood counts began to improve and graualy we were getting back the [redacted] we knew.

She continues to receive Eculizamab infusions every 2 weeks and all her blood test results contiue to be within normal limits with no sign of kidney damage.

If you could see [redacted] you would not believe that she has such a serious and life threatening illness and it is all thanks to Eculizamab. She is now leading a relatively normal 8 year olds life and all we want is for this to continue.

This drug was like a miracle and I beg you to consider making this amazing drug available for all aHUS sufferers as it is such a devastating illness for both sufferers and their families.

On behalf of [redacted] and her family I thank you for taking the time to read this.

Yours sincerely

[redacted]

[redacted]

In response to the announcement made by NICE on eculizumab for aHUS patients I would like to stress how much the eculizumab treatment has helped me since I was diagnosed with aHUS.

On the 24th December 2011 I was rushed into hospital on an emergency, I was violently ill and really thought I was going to die, I had no illness or symptoms leading up to this and cannot remember everything that happened as I was mostly unconscious but after some plasma and dialysis treatments I was diagnosed with aHUS and offered the eculizumab drug. It was nearly a month before I was allowed home after one treatment of eculizumab and booked in for further infusions of the drug. Since then I have been on fortnightly infusions of eculizumab and have never looked back, my life is back to more or less normal and it's all down to being able to have this wonderful drug, without this my kidneys would fail again. There are people in the same position as I was and many more waiting for transplants also needing eculizumab, and speaking from my own experience of aHUS I think it would be heatless not to allow these people the eculizumab drug, it's a wonderful drug and should be made available to anyone with aHUS.

[redacted]

NICE Decision on Eculizumab for aHUS Patients.

We felt compelled to write to you, following NICE's recent decision on the use of Eculizumab for aHUS patients.

My husband[redacted] received a successful live donor kidney transplant with aHUS in October 2013. [redacted]was diagnosed with aHUS after acute kidney failure at the age of 22. He was immediately treated with a course of plasma exchange, which, only proved successful for a few months, and resulted in chronic kidney failure, with[redacted]needing long-term haemo-dialysis. At the age of 22,[redacted]was still a full time farmer and was forced to give up his job, causing him to lose both his house and his job within a short time period. [redacted]poor health led to numerous complications, many of which required surgery and stays as an in-patient in hospital.

When we first heard of the introduction of Eculizumab, as a treatment for aHUS, we were so excited that this could result in a huge change in[redacted]life. For the first time, the chance of having a successful kidney transplant seemed a reality and then we were once again disappointed when Lord Howe decided to refer the AGNESS decision on Eculizumab to NICE. We fought to the highest level for[redacted]o receive this drug, allowing him to have a kidney transplant, including having a meeting with[redacted]from NHS England, to put forward our case. We were then thrilled and so relieved when Eculizumab was licensed in the interim period, until NICE had made a final decision. We were also fortunate enough for[redacted]to receive a live donor kidney transplant from my mother.

To now hear that NICE have decided to reject the use of Eculizumab for aHUS sufferers, seems to be such a backward move, and after seeing how well[redacted]has responded and recovered following his transplant seems well worth the cost of the drug. He is now healthy, fit and has regained his life with his family, work and lifestyle. The cost for treatment with Eculizumab, should not be allowed to outweigh the benefits to the patient and those around them who also suffer with their loved one's ill health. We recognise the high cost of Eculizumab, but strongly believe

that this drug has had such a life changing effect on [redacted] which could not have been repeated with any other form of treatment. [redacted] has many family members and three small children [redacted] who are all possible aHUS sufferers in the future and the thought that they will be unable to receive Eculizumab to prevent their long term kidney failure or the chance of a possible kidney transplant seems nothing short of disastrous. For them it will mean a life confined to long term dialysis and ill health, when in fact this need not be the case if Eculizumb is licensed by NICE. The inability to access a life-changing drug rivals the care of children in Africa, unable to receive quality medical treatment and should not be the case in the western world.

We are standing strongly alongside aHUSUK to support and appeal for the license of Eculizumab for aHUS suffers and will not rest our case until NICE recognise and reinstate the use of Eculizumab for aHUS sufferers on a national scale.

We hope that you will reconsider the decision made and work with Alexion to find the best way forward for using Eculizumab to treat aHUS.

Many thanks for taking the time to read our letter.

[redacted] (aHUS patient with kidney transplant)

[redacted]

Dear[redacted]

I'm responding to the announcement made by NICE regarding eculizamab for Ahus patients.
Our 3yr old daughter[redacted]was diagnosed with this horrible disease 2 weeks before her 1st birthday, she became very ill very quickly.

It started with diarrhea but she then started having seizures that lasted for hours! Things became very serious and she went into kidney failure, we nearly lost her but thankfully she was diagnosed and treated quickly. She spent 2 weeks on daily dialysis where she was so poorly she would just lay in her cot, not crying or really moving. She was then started on the wonder drug eculizamab and within 2 days we had our little girl back! The difference in her really was miraculous.

[redacted]has been receiving eculizamab ever since. She has a dose every 3 weeks, which the hospital hopes to stretch to every 4 weeks after our next visit.

The thought of our beautiful little girl having to endure hours of dialysis or plasma exchange on a daily basis with the possibility of the end result being death fills us with dread and fear. It’s hard to understand how a drug that allows our little girl to lead a normal happy life and that is already available in this country for use of another disease can be denied.

We feel Ahus patients are being treated very unfairly and hope that this emotional roller coaster will soon come to an end when the right decision is made.

Yours sincerely
[redacted]

Response to NICE announcement on eculizumab for aHUS.

I am [redacted] an aHUS patient who has been on Eculizumab for two and a half years. I am a member of aHUSUK and [redacted] However I am writing this in my personal capacity to tell the evaluation committee how hugely effective Eculizumab has been for me.

I was diagnosed with aHUS three years ago. We had never heard of the disease and the more we found out about it the more devastated and scared we became. We couldn't believe that in the space of three weeks I changed from being a healthy 63 year old, living an active life, to lying in a hospital bed with kidney failure requiring plasma exchange every day to keep me alive. I remember being keen to have this treatment but at the same time dreading it as I suffered a variety of disturbing side effects during almost every session. I was dealt a further blow when my then PCT refused an application for funding for Eculizumab, a drug I was told would improve my condition considerably and was much less invasive, on the grounds that my case was 'not exceptional'. That was hard to accept when at that time there were only 170 people in the UK with this ultra rare condition. Daily plasma exchange continued for the seven weeks I was in hospital, then when it was reduced to every second day I was allowed home. I left hospital in a wheelchair and at home I was totally dependent on my husband. Over the next few months the frequency of plasma exchange was gradually reduced until I was receiving it fortnightly. By then I was a bit stronger but I was still greatly restricted in what I could do. I lived with the constant worry of the 'central line' being infected and the threat of the plasma exchange ceasing to be effective and having to become dependent on kidney dialysis or worse. The future was very uncertain.

However five months and 65 plasma exchanges after diagnosis I was fortunate enough to be included in a clinical trial for Eculizumab. After the very first infusion I felt my condition improve slightly and within a few weeks I started to live again. The 'central line' and with it the risk of infection were removed. I became stronger, grew in confidence and quickly increased my activities. I felt I had a future again. Because of the delay in receiving Eculizumab I have considerable irreparable kidney damage but Eculizumab has restored sufficient kidney function for me to lead a normal life. Now between the fortnightly infusions I go home and get on with my life. After two years and six months on Eculizumab I feel so well and can do so much that I sometimes find it hard to believe that I have this devastating, life-threatening ultra-rare disease. I cannot stress enough the enormous difference Eculizumab has made to me and my family.

During the first few weeks of my illness when I didn't respond to the first type of plasma and I continued to deteriorate it was heart-breaking to watch my sons, who live away from home, leave hospital on a Sunday night. Each week I wasn't sure I would see them again. However I have now enjoyed both their weddings and have recently met my first grandchild!

Eculizumab has given me my life back and we as a family now have a future together. I would like that for all patients who, through no fault of their own, are unfortunate enough to develop aHUS.

[redacted]

Dea[redacted]

I wish to respond with regard to the recent unsatisfactory decision from NICE in relation to Eculizumab and its treatment of aHUS sufferers. Our daughter became seriously ill out the blue in February 2012 aged 16yrs. She suffered renal failure and spent the next four months in hospital and 18 months on haemodialysis 3 x weekly for 4 hours/session. She had frequent shorter stays in hospital throughout that time too.

[redacted] was diagnosed with HUS, we were told this was not genetic and not likely to recur though the damage was done. Between Feb 2012 and end of August 2013 she endured multiple medical procedures. She had many central lines put in, finally requiring surgery for a fistula. She contracted severe pneumonia which resulted in a stay in ICU under sedation and on a ventilator. Her heart was affected. She lost so much weight through vomiting and inability to eat that she had to be tube-fed for two months. She endured bronchoscopy, biopsies, endoscopy and constant blood tests. [redacted] lost so much time at school she was unable to return and complete her A Levels and had to change her path altogether. Her dad and I have suffered financial difficulties due to loss of earnings while supporting [redacted] through those terrible days. The stress and strain on the family has been huge.

Having already felt we were losing our young daughter on a couple of occasions, matters worsened on 23 December 2012 [redacted] suffered unbearable pain in her head. As I drove her to hospital she suffered a massive seizure (she is not epileptic). I had to wait too long for an ambulance while my barely conscious daughter lay sprawled across my lap. She proceeded to take multiple seizures. Having no awareness or diagnosis of aHUS, Cumberland Infirmary in the best of their wisdom heavily sedated [redacted] and she spent Christmas week in ICU once again on a ventilator. They carried out lumbar puncture and other tests but still no diagnosis of aHUS. [redacted] was told by a renal consultant that the seizures (resulting from excessively high blood pressure - over 200) were due to a rare reaction to the dialysis and 'would not happen again'.

[redacted] was called for transplant at the Freeman in Newcastle end of August 2013. All went well until a few days after the transplant when [redacted] blood pressure soared excessively once more, she was screaming with the pain in her head, and began once more to have seizures. We had to stand by and watch as our daughter lost much of her vision, and the feeling in her arms and legs. She was unable to recognise medical staff she knew well, and once again we thought she was going to die. Fortunately for us [redacted] and his team were on hand the whole time and arranged access to Eculizumab for [redacted] as quickly as they possibly could. While this was going on in the background my husband and I waited to lose our little girl. Words cannot express the fear this type of situation puts you in. A fear that can still be felt just by thinking about that day. The tears that come into my eyes each time I remember, sitting by my daughter's bedside helplessly, her reaching her hand out to me and whispering 'I love you mummy' and my knowledge that she too was in fear of her life.

[redacted] was taken to ICU and administered her first dose of Eculizumab that night. We were terrified and did not hold out much hope. On seeing [redacted] the following morning after the Eculizumab the difference was night and day. She was bright and calm and pain free. We felt like we had been given our daughter back. It was like a miracle to us, an absolute miracle.

Without Eculizumab [redacted] at best, would have lost the new kidney and ended up back on dialysis. At worst, she would not be here today. Now a bright 18yr old who is now able to apply for universities near London for this September and pursue her dream of becoming a journalist. She continues to have ups and downs with other medications relating to the transplant but the Eculizumab has been like a new lease of life, for her and the family. We still worry about her but now she has the diagnosis of aHUS and is on Eculizumab we feel hopeful for her future and reassured by the care she is under at the Freeman. Thanks to the wonders of Eculizumab [redacted] now has a social life, and energy, and all the things most people take for granted.

The use of this valuable life saving and life giving drug should not be hampered in any way.

Yours sincerely

[redacted]

Dear[redacted]

I write to you as I understand that you have involvement in the current issue of the possibility of Eculizumab being made available to all individuals affected by Atypical Haemolytic Uraemic Syndrome. I ask that you pass my brief report on to whomever is appropriate. I am writing from my work email address as I understand that only Microsoft office emails are successful in reaching your account at this time, but I write as an individual.

My baby son [redacted] was struck down "out of the blue" with aHUS (later found to be secondary to a Factor H mutation) in November 2009, when he was aged only 6 months. His life was at risk for some time, and he had neurological involvement, but he eventually responded to daily plasmapheresis (at the time, the only treatment available) and haemodialysis. Due to the early intervention, his kidneys recovered enough function to manage without dialysis, but he remained completely dependent on plasmapheresis between one and three times every week for nearly three years. Living in Exeter, and the nearest treatment centre being in Bristol, this involved a 12-hour round trip each time (four hours travelling time, waiting time, four hour treatment time). I was unable to work during this period, and my older child often had to attend Bristol Children's Hospital School. Every time [redacted] doctors tried to lengthen the gap between his plasmapheresis sessions, he went into relapse. [redacted] life (and that of his family) completely revolved around his treatment regime. Furthermore, having a vascular catheter in situ, [redacted] activities were greatly restricted – including not being able to engage in any water-play, swimming, or even bathing. He also suffered "line infections". [redacted] was severely developmentally delayed in his gross motor skills and speech and language skills. [redacted] consultants applied to the appropriate NHS Trust when he was two for funding for eculizumab treatment, but were turned down. A year later, they re-applied as an "emergency application" when his "line" finally failed. This time, they were successful. The impact on [redacted] life and that of his family has been dramatic. For the past 18 months, [redacted] has only had to attend hospital for treatment once every two weeks in Exeter instead of Bristol – and the session only takes half a day in total. As a result, he now lives a much more "normal" life and is able to enjoy many "normal" activities as he no longer needs a vascular catheter (with two lumens). He does now have a portacath (since cannulation became impossible) but, of course, being internal, this does not affect his activities to the same extent as an external vascular catheter.

Since [redacted] has been receiving eculizumab treatment, he has not suffered even one relapse of aHUS symptoms. His developmental rate has accelerated and, since starting full time school (Reception) in September, he has learned to read, write, jump and speak in proper sentences. We have all been amazed, as he did not speak a word or walk until he was over two years of age. Last month, he was even discharged from the Speech and Language Therapy Department! I am convinced that this huge improvement is partly due to the direct and indirect effect of eculizumab treatment over plasmapheresis treatment (i.e. direct effects include him having been "released" to spend more time doing "normal" "developing" activities instead of travelling to and from Bristol and being hooked up to a machine for hours and hours on end). I have also been able to return to work.

If [redacted] had to return to his old treatment regime, I genuinely believe that the effect on him would be catastrophic, and severely detrimental to his entire family's functioning and wellbeing.

Furthermore, had [redacted] received eculizumab when he first succumbed to this devastating illness in 2009, I fully believe that he would not have suffered such serious long term effects from the initial episode (his kidney damage was such that he has suffered permanent issues with difficult to control hypertension, intestinal damage, neurological damage, left ventricular hypertrophy and arrhythmia etc etc), and subsequent relapses. It seems unbelievable that not making this life-saving, life-transforming drug available to all aHUS sufferers automatically can even be considered. Surely, a price cannot be put on any of these innocent victims' lives?

I submit this for your serious consideration.

Yours sincerely,

[redacted]