Appendix 2 – NRAS short case studies and free-text responses from survey (Appendix 1)

Appendix 2 forms part of the NRAS Response to DSU Report and Assessment Report relating to the Multiple Technology Appraisal (MTA) Adalimumab, etanercept, infliximab, certolizumab pegol, golimumab, abatacept and tocilizumab for the treatment of rheumatoid arthritis (review of TA guidance 130, 186, 224, 234 and part review of TA guidance 225 and 247) [ID537]

[redacted]

I am a 46 year old female who was diagnosed with RA in 2002, put on a combination of Methotrexate, Prednisalone, Calcichew, pain killers and Etodolac. After 5 years it became apparent that the Methotrexate wasn't doing its job so I was then given Sulphasalazine in its place. This caused me excessive weight loss so was eventually replaced with Leflunomide which I have been taking for some 5 years plus now, I believe. Due to not successfully controlling my symptoms, I was prescribed Hydroxychloroquine to take alongside the Leflunomide, (and other meds), which brings my story up to date. I have failed DAS scores on several occasions in the past, but have raised DAS 1 month ago and am due to have a second DAS done tomorrow with a view to putting me forward for Biologic drugs asap.
I returned to an office based job on a part time basis two years ago after being out of work due to my illness for almost 11 years. My main fear is that I am now deteriorating again and I may have to give up work again. I do not want to go backwards.

[redacted]

I was diagnosed with Rheumatoid Arthritis in November 2014, after my symptoms got progressively worse since 2013. I was originally told it was my weight and made to feel I was wasting a doctors time, but after asking for a second opinion and blood tests it was diagnosed as RA.

I was started on DMARDS but was allergic to them and so then given Methotrexate which made me very breathless, after x-rays I was taken off them and put onto steroids for 6 weeks. My consultant now seems to think as I have no damage to my joints at present, I can manage on 3 steroid injections a year. I am not allowed a biologic as it is too expensive and I suspect as I am 67years old !!!!!

So regardless of the pain which is excruciating at times and continual fatigue I am on these injections which put weight on the already big weight problem I have. Yes if I was allowed

this biologic and it gave me a better quality of life I would go for it.

In 2 years I am in a wheel chair as cannot walk, am now finding my arms cannot cope with driving, I need help to dress and I cannot perform the basic cleanliness tasks. I cannot lift the kettle, you name it feels I cannot do it, do these “professionals” know how frustrated and useless one feels???

Over the last two and a half years I have tried Sulfasalazine, which made me very sick. I have been on the maximum dosage of Hydroxychloroquine for 19 months, with no benefit. I have also been taking Methrotrexate since September 2014. The tablets made ill, and now injection dosages have increased from 10mg through 15mg and 20mg, with no effect except to make me very tired. I am in constant pain, and take painkillers every day. I walk with a stick and then not more than 50 yards. My world is very small; I cannot travel or do normal day to day activities.

RA started in one ankle joint, and DMARDS have done nothing for me. RA is in fact now spread to both ankle joints, making my mobility so poor. NICE will not recognise RA in feet and ankles as part of the DAS. This is totally unacceptable. I hope that my consultant will apply for biologics for me, given my situation. If I cannot access biologics I do not see what my future treatment can be. I will be failed by the NHS.

I have been diagnosed for approx. 2 yrs, I am currently on 25mg methotrexate injection weekly, hydroxychloroquine 4oomg and 2000mg sulphasalazine daily, these drugs make me feel worse than the actual disease and I am ready to give in, I work 5 half days a week and have to inject methotrexate on Friday as I am ill all weekend.

I am 42yrs old with 2 teenage boys and my husband who I try to take care of, I work as a receptionist in a busy Doctors surgery during the week. In an ideal world there would be something out there for me that slowed the disease without destroying me! I am well looked after by the nurses but have only ever seen a consultant on the occasion of my diagnosis and have no idea what my das score is, I just know I am yet to be in remission.

I am aware that I have not been in this system very long so not sure if my story will be useful or not, but in my opinion these drugs are awful and reduce quality of life rather than offer the improvement that is hoped for.

I would like to say that the drug rituximab saved my life, I was at the stage with my RA where I could not stand the pain anymore after 10 years and all the drugs that my team tried did not work. I could not walk, I just laid on the sofa and cried, all the time filling my body with painkillers as if they were sweets. I was fat with all the steroids that were pumped into me, constantly having urinary tract infections and all the time having antibiotics and just losing the will to live, Then a miracle happened, and that's what it was a MIRACLE, my RA team said I qualified for rituxamade, and my life changed completely , I have had three sessions spread out over three and half years, and I know I could not go back to the place I was in before, ok I have flares occasionally which I can cope with, I have lost weight as I am more mobile, no more infections, Surely the cost of this wonder drug, far out weighs the

cost of me having to be constantly at the GPs and hospital, being supplied with antibiotics and masses and masses of painkillers, plus the thought of a heart attack due to weight gain. Please please I beg, not just for me but for other poor people with this debilitating disease to let us continue with it.

I was diagnosed with Rheumatoid Arthritis in 2009. I was put on a number of drugs including Methatrexate over the following months but while things improved for a while, the pain, fatigue, difficulty in mobility and lack of strength returned. The impact on everyday life was significant for me, with the pain in my wrists being the biggest obstacle. With Grandchildren entering my life I wanted to play an active role but hated the idea I wouldn't be able to pick up a baby.

In 2011 I was put on a Biologics treatment, Humira. Apart from a fusion operation of two joints in my foot in 2013, which was a great success, I am currently in remission. I work 15 hrs a week, enjoy my time with my Grandchildren and can enjoy a bit of "pottering" in the garden (no heavy work). By managing my activities and rest times sensibly along with the drugs and Biologics I am very happy and very grateful.

If I was no longer receiving Humira or it stopped working I would dread the return of the misery of painful joints, stiffness, fatigue and lack of wellbeing I now have.

In November of 2013 My consultant at Lincoln County Hospital decided that after being on methotrexate (controlling RA to a certain extent), and sulphazasaline (very little impact on my RA) that my RA was not being controlled adequately. I was at this stage suffering more flares and flares in different joints especially my feet, which was completely debilitating causing me to have to crawl on my hands and knees.
I started Enbrel in February 2014. This was absolutely my wonder drug. Working very quickly on the inflammation which also improved the fatigue side of RA. Sadly I was having allergic skin re-actions to this and had to come off. Whilst waiting for funding for Humira I had the worse flare ever, so in answer to your question about biologics being withdrawn. I can from personal experience say that the affects are quite horrendous.

Humira has given me my life back. I still pace myself as I have damage from RA but believe I am now in re-mission because of the Humira though understand I have to be on these type of drugs to keep myself in remission. Hope I will be one of the lucky ones that Humira will stay working for me.

I was diagnosed with palindromic rheumatoid arthritis 2 years ago after experiencing shifting joint pain and extreme fatigue. I was 68 at the time. I was put on DMARDS but experienced an allergic reaction. Because of an underlying kidney problem I was unable to go on to methotrexate so I was kept on sulfasalazine for some time with little improvement, and had to have short courses of steroids to get me through the worst times.

As my DAS remained high and I was still suffering from joint pain (albeit now localised more to my wrists and fingers) I was cleared to go onto biologic drugs about 9 months ago. Initially the improvement was not marked and I had to have another course of steroids. However in the last 2 months things have really improved and I would say that I feel back to how I felt before developing RA.

I have some residual wrist pain but it does not really affect what I can do. Although retired, I now feel able to resume activities that were impossible a year ago (gardening etc), and my quality of life is very good. I still have a degree of fatigue bit it is felt that this may be more due to my kidney problem than due to RA.

I consider myself to be in remission now and would dread the thought of not being able to continue with biologics.

[redacted]

My name is [redacted], aged 22, and I have recently been started on biologic therapy in combination with methotrexate, for the treatment of my arthritis. I am 5 weeks into the treatment and have already noticed a huge improvement. I can now lift mugs and plates with one hand with hardly any pain and the swelling in my fingers and knees has mostly subsided. I no longer get severe wrist pains in the morning and can start the day feeling more positive! I don't know what I would do without this medication as it has already made such a huge difference and I can't wait to see even further improvements. I can't imagine going back to DMARDs alone and would be devastated if I had to live my life this way again. I feel younger and more like a normal 22 year old (despite the strange looks I get down the pub when I show people that I can now lift chairs) and have more hope for the future. Despite this, biologics come at the price of being more prone to infection, however I'm willing to tolerate the occasional bouts of tonsillitis in exchange for having my life back.

[redacted]

I began not to cope with life. I was permanently exhausted. My body hurt. My hands and wrists didn't work. My fingers curled over and would not straighten. My feet would not co-operate with shoes or walking. My knees were swollen and painful. My neck didn't turn without extreme pain. I was clumsy and angry with myself. What was happening to me? My mum died and I had three months off work. The Doctor gave me Prozac.
I was eventually diagnosed with RA. I had 20mg of Methotrexate and a Steroid injection. There was some improvement for about six weeks. I had Steroid injections in my fingers and Hydroxychloquine was added. I was still not able to look after myself. I can still vividly remember being on my own in the house stuck half in and half out of a tshirt. I thought I could undress myself. I went back to work. I took Diclofenac and Cocodamol to get me through the day. I had an outstanding school and I wasn't good enough. I retired.
I was given Tocilizumab as part of a Clinical Trial. I improved almost immediately.
I could walk and drive again. I could choose what I wanted to wear, except heeled shoes. My feet are still unco-operative. I could garden and cook and look after my partner. I go to yoga classes twice a week. I grow lots of vegetables and flowers. I go on lots of holidays. I am on the local Patient Partnership Group.
I still have problems but they are manageable. I would not want to go back to how I was

before the biologic drug. I was quite helpless and needed support from others. Now I can look after myself and my family.

I was diagnosed in February 2012 with RD. I was very unlucky and had it in practically every joint practically over night. I couldn't get out of bed and had to be carried to the bathroom. I was put on steroids which helped greatly and started on meth and HCQ. Both of these helped but as soon as the steroids were lowered I was in agony again. Couldn't walk with out a limp and in constant pain. Couldn't use my hands.
March18 2013 .at 6.30 pm ,first injection of Enbrel, by 11.30 pm I got up out of the chair like a normal person, (without having to launch the top half of my body forward to drag the lower half of me out of the chair, as my knees and ankles couldn't help me). then I walked half way up the stairs and realised I wasn't putting both feet on each step and leading with the right leg (as this leg was less painful) and came down the stairs fairly normally. I was excited but knew the test would be coming off the steroids. Well I went from 25 mg prednisolone to none and yes the Enbrel is still working. I have a few side affects but nothing I can’t live with. I have my life back. I still have the odd twinge if I walk or stand for hours. I get a few chest infections, then I have to stop my injections and take antibiotics and the pain starts to come back and I can’t wait to start my injections again.
I truly have my life back.

I have been in Enbrel for 3 months now and it has completely changed my life. I can now do most things I did before developing RA, some 3 years ago.

I was ill for a year before diagnosis and then 2 years on DMARDS, including sulfasalazine, methotrexate and leflunomide. Nothing worked effectively and I had to reduce my working days, stop hobbies etc. I had pain every day and had to be helped to dress, bathe, walk. I noticed major improvement with my first injection of Enbrel 3 months ago and since then have continued to improve.

I would be completely devastated if I couldn't continue on the biological drugs. My life would not be worth living if the Enbrel was taken away. This drug has literally been a life saver for me; I was so depressed because of the pain and limitations in my life that I seriously considered ending it.

I was diagnosed with RA aged 27. At first I was on Sulfsalazine and Methotrexate. I stayed on that combination for 2 years. I didn't find it helped and the Methotrexate made me feel quite ill for 6 days out of 7. I had a poor quality of life. The Methotrexate was replaced by Hydroxyquinaline and I was on that combination for a year with little or no improvement.

My consultant said he'd like to try Etanercept. This was a revolution in my treatment. Within weeks I was feeling less pain, less fatigue and had more movement. I was on Etanercept for a good while but unfortunately the one style injections did not suit me and I started to become needle-phobic. My consultant decided to put me on Infliximab as it was done via infusion and would be less stressful for me. Unfortunately, after 4 infusions, I took

anaphylaxis reactions to infusions 5 and 6 so had to discontinue. The few short months on Infliximab had made such a massive improvement on my life. I have now been on Golimumab for nearly 2 years and have achieved remission status with occasional breakthrough flare up. I can go hill walking, mountain biking, road cycling, snorkelling, etc. I can be a proper, active Mam to my son. For me, Biologics have given me my life back.

I was diagnosed with a severe and unremitting form of RA in 1991. I had to give up my work as a lecturer only 6 months after diagnosis and the following year, had the first of many joint replacements and surgical interventions.
It took time to find the optimal biological treatment and I was extremely grateful to be granted access to a number of therapies before - miraculously! - finding the one which works for me. I've ceased to be a wheelchair user and, although I continue to live with severe cervical and lumbar spine damage, I have regained some useful mobility and, most importantly, a valuable degree of energy after years of exhaustion and fatigue. (I feel strongly that it is often the latter which impedes individuals from living and working to their potential.)

It would be no exaggeration to say that, after trying other biologics without any improvement, access to an effective therapy has changed my life. I undertake voluntary work for several charities and I would like to think that the personal fulfillment which I gain is complemented in benefits to the organisations for which I volunteer.

I qualified for tocilizumab infusions about 18 months ago because I failed on other drugs. By this time the disease had progressed rapidly and I was in a great deal of pain in both wrists, fingers in both hands, thumbs in both hands, right elbow, right shoulder, left knee, both hips, left ankle, left toe joint and left big toe. I was considering taking early retirement or going part time because of the impact on my job, and my social/hobby life pretty much stopped. Everything I love doing - sewing, jewellery making, photography, finger weaving, micromacrame and other hand-intensive hobbies - I had to stop. I had no energy, I was sleeping over 10 hours a day, my mood was depressed and all I could see was doom and gloom. I couldn't drive because of the pain in my feet and I was more or less housebound. I shuffled around like a 90 year old.

My reaction to the infusion was slow. There were some small but noticeable improvements after the first infusion but it took the second infusion to really make a difference. By the third infusion I had gone back to using one walking stick, I could drive, I could go back to my hobbies, I had no more hip pain and all previously affected joints reduced in size and pain levels. My energy levels were higher than they'd ever been and I was sleeping better. My mood was elevated and I went back to being my usual cheerful, cheeky, optimistic and challenge-loving self. I actually began to forget to take pain medications and wasn't watching the clock for my next dose as previously. In short, I got my life back.

After a year we trialled weekly injections at home. I found the subcutaneous injection mostly ineffective and within a few weeks I had gone back to how I was before the

infusions. The disease progressed and caused what appear to be permanent problems in my left foot, meaning I now have to buy an automatic car. I was returned to infusions and within a couple of weeks of the first infusion things improved rapidly again. I'm driving again using a manual transmission and am able to go to work, go out and have a normal life.

Tocilizumab has changed my life. Without it I would be mostly disabled by now and in a tremendous amount of pain. I wouldn't be working and I would be pretty much housebound without an adapted car (no foot pedals). My life would be horrible. I've had - for the very first time - barely any side effects other than some weight gain and a small rise in cholesterol levels which I'm managing through a vegan raw/Mediterranean diet which has made an amazing difference to my health overall.

[redacted]

I have had RA since 1986 and have had a few flare ups over the years. Four years ago I was on Methotrexate, Sulphasalazine and Hydroxychloroquine and doing well until the Doctors suggested that I drop off the Sulphasalazine. This caused a bad flare up which didn't respond to restarting Sulphasalazine or increasing my methotrexate dosage. My joints were badly swollen, I was in a lot of pain and my company tried to get rid of me on the grounds of poor health even though I was doing my job to the required standard.

I was put onto Enbrel and it was a revelation for me. I felt the difference within a week, was much better after two weeks and after six weeks the swelling in my joints had gone and my pain levels were almost zero. I was once again able to do the things I could before the flare up started, Enbrel gave me back my life as I know it. I work full time, pay 40% tax and believe that I make a valuable contribution to society. If Enbrel were to stop working and I was refused another biologic I would prefer death to a life of pain and misery.

[redacted]

I developed rapid onset rheumatoid arthritis at the end of 2011 aged 65.
I had been an active person-gardening, hill walking and travelling the world before the arthritis struck. The arthritis left me unable to walk properly, get myself up from a sitting position or stand for any length of time. DEMARDS helped me to do these everyday things again but with difficulty and pain. I certainly could not carry out my former activities. After being accepted for the biologic CIMZIA my life is back to normal and I am virtually pain free. I have just returned from Australia to see my grandchildren, something I could not have contemplated before taking CIMZIA. If CIMZIA or an equivalent biologic was withheld, my quality of life would be considerably reduced. It doesn't bear thinking about.

[redacted]

"The rheumatoid arthritis (RA) is better controlled since I began taking the biologic therapy Cimzia in October 2014. My life is heading in a positive direction. There are a number of ways that the biologic has had a positive impact on my life: (1) I can resume looking after my family and home and keeping in touch with friends; (2) I thought I was going to have to stop working but now I can continue with self-employment and voluntary work projects; (3) since RA I had to stop running, playing tennis and taking part in aerobic classes, I can now begin to build up my muscle strength - going to the gym and walking as well as attending tai chi classes for relaxation; and (4) I have been able to reduce or cease the other drugs I was taking so now suffer less side effects. I would feel extremely concerned if I thought I had to

go back to the pre-biologic days of lack of mobility, walking with a stick, sore, painful and swollen joints, sleepless nights, fatigue, nausea, anaemia, depression and tearfulness, lack of self-confidence and fear about what was happening to me."

[redacted]

After reading your request on the Healthunlocked forum I felt I had to write a little about me and my RA. I am truly horrified to read that there is a chance that if for some reason I have to stop taking Enbrel I may not be offered another biological drug. I know that funding in the NHS is constantly in the media but surely someone should realise that if we are denied these life changes drugs we would be more of a burden on the state than the cost of the drugs. More hospital admissions, joint replacements, not to mention the help that we would be entitled to such as PIP etc.
After going through the strict criteria set down by NICE I started Enbrel in November 2012. My RA at this point was really bad. I had a DAS score of 7.2 at my first assessment, heaven knows what it would have been by the time I started it. I was more frightened of the drug not working than any side effects. I felt the benefit of the drug the next day and at my three month assessment I was in remission and to date I still am. No pain relief needed at all!

This drug simply gave me my life back. It gave me back something that this terrible disease took away. From feeling like a worthless blob sitting on the sofa, in pain every time I moved I am now full of energy, pain free and able to live life to the full....truly an amazing drug.

I know that there is always the possibility of the drug not working but I have always felt safe in the knowledge that if this did happen there were other options for me to try. To think that this option could be taken away from me truly horrifies me. Going back to the black place is somewhere I just don't want to return to.

A selection of anonymous free-text responses from our survey (Appendix 1)

Question: If you are receiving biologic therapy, how would you feel if you no longer had access to this medication or another biologic therapy in future? What impact would this have on your life?

Quality of Life

"It would be devastating. My life would not be worth living I would probably be in constant severe pain and unable to function."

"I would be horrified if I no longer had access to biologic treatments as I know that my disease would become completely out of control rendering me completely immobile and incapacitated as well as in severe, continuous pain."

"I would feel suicidal, if my arthritis returned to pre-Enbrel levels it would ruin my life, leave me unable to work or maintain any quality of life. Suicide would genuinely be the first option I would consider."

“I would have to stop working as an Advanced Nurse Practitioner. I would be totally devastated. The terrible debilitating fatigue and stiffness would return. The pain in hands feet knees ankles hips shoulders would return. I would not be able to sleep through the night even with anti-inflammatory and analgesia. I would become depressed again. I would lose hope I would lose interest in life.”

“Terrified. Humira transformed my life. Previously I was unable to live a normal life. Pain often too severe to think of anything else. Often unable to dress. Usually unable to prepare food, do up buttons, tie shoelaces, write, etc.”

Work

“If the Rituximab was removed I believe that I would not remain in employment. Even with this therapy I struggle to remain at work because of the fatigue and recent deformity of my dominant hand.”

“I would be devastated if I could no longer have biologic therapy. When first diagnosed I deteriorated quickly and was in a wheelchair and unable to open door, dress myself, shower unaided etc . As a result of the drug regime my swollen, painful joints once again worked normally and I was even able to return to work full-time as a primary school teacher (I am now retired because I am 66).”

“I would end up losing my job and my life as I know it. My RA is getting worse each year but is being controlled well with my RA team but if I lost the meds I would lose everything, I wouldn't even be able to get out of bed.”

“I would be terrified, I am on a biologic because this was the first treatment that kept my disease under control. I live alone and support myself by working full time, if my health deteriorated it's unlikely I would be able to work full time. I do not know what my future would hold, I feel sick just thinking about it.”

Family

“Recently taken off Etanercept which appeared to be fantastic compared to Rituximab which I have just been started on. During the changeover time I was virtually unable to walk, with severe pain and swelling in my knees hips and shoulders, impacted severely with caring for my children and also affected my marriage due to my husband having to take a lot of the home responsibilities on.”

“I'd be devastated as I have not tolerated DMARDS or benefitted from them so biologics are my only hope of controlling this disease and living a relatively normal life. I want to be an active part in my children's lives and enjoy life without the restriction of pain and stiffness and the unrelenting fatigue.”

“I would be unable to care for my 2 young children, I would be unable to continue my part time employment and I would need help with everyday tasks.”

Wider impact on society

“We are a close, loving family but I would feel like a burden. There would be more strain on my husband who does a great deal for me already. I would have to apply for help from the

state thereby costing more to the taxpayer. I can't even imagine how I would cope as the pain is unbearable during a flair."

"At the moment I work full time, pay 40% tax and make a valuable contribution to society. Without Enbrel, or the right to try a second biologic were it to fail, I feel that I would be being 'written off' as a human being and considered 'beyond economical repair'. This would condemn me to a life of pain and misery, I would have no quality of life and in such circumstances would frankly rather be dead than suffer. This may sound dramatic but anyone who's been through a flare up themselves or has seen a loved one go through it would understand why I feel this way. Enbrel gave me my life back as I know it and the thought of life without it fills me with despair."

"It would be unbearable, I would be disabled. Although I am unable to do the travelling consultant and trainer role that I did before RA hit me, I am able to work from home as a writer. Although now on a very low income, I do not draw money from the state. Without biologics, I would be completely dependent on the state."

"I would be unable to work, would lose my house, would have no independence or income, and would be on benefits. In addition I would need far more support from health professionals for pain, and systemic symptoms such as anaemia. My ability to walk would be affected and I would have trouble looking after myself. I would become very depressed and isolated, as I would not be able to walk my dog and would have to move from the area I currently live in, where I have social support and neighbours."